This short form base shelf prospectus has been filed under legislation in each of the provinces of Canada that permits certain information about these securities to be determined after this short form base shelf prospectus has become final and that permits the omission from this short form base shelf prospectus of that information. The legislation requires the delivery to purchasers of a prospectus supplement containing the omitted information within a specified period of time after agreeing to purchase any of these securities, except in cases where an exemption from such delivery requirements has been obtained.

A registration statement relating to these securities has been filed with the United States Securities and Exchange Commission but is not yet effective. These securities may not be sold nor may offers to buy be accepted prior to the time the registration statement becomes effective. This prospectus shall not constitute an offer to sell or the solicitation of an offer to buy, nor shall there be any sale of securities in any state in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state.

No securities regulatory authority has expressed an opinion about these securities and it is an offence to claim otherwise. This short form base shelf prospectus constitutes a public offering of these securities only in those jurisdictions where they may be lawfully offered for sale therein and only by persons permitted to sell such securities.

Information has been incorporated by reference in this short form base shelf prospectus from documents filed with securities commissions or similar authorities in Canada and with the United States Securities and Exchange Commission. *Copies of the documents incorporated herein by reference may be obtained on request without charge from the Vice President, Finance of BELLUS Health Inc. at 275 Armand-Frappier Boulevard, Laval, Quebec H7V 4A7, Tel: 450-680-4500 and are also available electronically at www.sedar.com. See "Documents Incorporated by Reference".*

SHORT FORM BASE SHELF PROSPECTUS

New Issue and or Secondary Offering January 17, 2020

BELLUS HEALTH INC.

US$250,000,000
Common Shares

This short form base shelf prospectus relates to the offering for sale from time to time, during the 25-month period that this prospectus, including any amendments hereto, remains valid, of common shares of BELLUS Health Inc. (the "**Company**"), with a total offering price of such securities of up to US$250,000,000 (or its equivalent in any other currency used to denominate the securities at the time of offering). The securities offered hereby may be offered separately or together, in separate series, in amounts, at prices and on terms to be set forth in one or more prospectus supplements. One or more shareholders of the Company may also offer and sell our common shares under this prospectus. See "*Selling Shareholders*" and "*Plan of Distribution*".

All shelf information permitted under applicable securities legislation to be omitted from this prospectus, including, without limitation, the information disclosed in the specific terms of any offering of securities, as discussed above, will be contained in one or more prospectus supplements that will be delivered to purchasers together with this prospectus, except where an exemption from such delivery requirements has been obtained. Each prospectus supplement will be incorporated by reference into this prospectus for the purposes of securities legislation as of the date of such prospectus supplement and only for the purposes of the distribution of the securities to which that prospectus supplement pertains.

We are a Canadian company incorporated under the *Canada Business Corporations Act*.

The Company is permitted, under the multi-jurisdictional disclosure system, or "MJDS", adopted by the securities regulatory authorities in Canada and the United States, to prepare this prospectus and any prospectus supplement in accordance with Canadian disclosure requirements, which are different from those of

the United States. Financial statements included or incorporated by reference herein have been prepared in accordance with International Financial Reporting Standards, or "IFRS", as issued by the International Accounting Standards Board, or "IASB", and may not be comparable to financial statements of United States companies. The Corporation's financial statements are subject to audit in accordance with Canadian generally accepted auditing standards and/or the standards of the Public Company Accounting Oversight Board (United States), or "PCAOB", and our auditor is subject to both Canadian auditor independence standards and the auditor independence standards of the PCAOB and the United States Securities and Exchange Commission, or the "SEC".

The enforcement by investors of civil liabilities under United States federal securities laws may be affected adversely by the fact that we are incorporated under the federal laws of Canada, that most of our officers and directors are residents of Canada, that many of the experts named in this prospectus may be residents of Canada, and that most or all of our assets and the assets of said persons are located outside of the United States. See "*Enforcement of Judgments Against Foreign Persons or Companies*".

THESE SECURITIES HAVE NOT BEEN APPROVED OR DISAPPROVED BY THE UNITED STATES SECURITIES AND EXCHANGE COMMISSION NOR HAS THE SECURITIES COMMISSION OF ANY STATE OF THE UNITED STATES OR ANY CANADIAN SECURITIES REGULATOR APPROVED OR DISAPPROVED THESE SECURITIES OR PASSED UPON THE ACCURACY OR ADEQUACY OF THIS PROSPECTUS. ANY REPRESENTATION TO THE CONTRARY IS A CRIMINAL OFFENSE.

The specific terms of any offering of common shares will be set forth in the applicable prospectus supplement and may include, without limitation: the number of common shares being offered, the currency (which may be United States dollars, Canadian dollars or any other currency), the offering price (in the event the offering is a fixed price distribution) or the manner of determining the offering price(s) (in the event the offering is not a fixed price distribution) and any other specific terms. A prospectus supplement relating to a particular offering of securities may include terms pertaining to the securities being offered thereunder that are not within the terms and parameters described in this prospectus. Where required by statute, regulation or policy, and where the securities are offered in currencies other than Canadian dollars, appropriate disclosure of foreign exchange rates applicable to the securities will be included in the prospectus supplement describing the securities.

The securities may be sold to or through one or more underwriters or dealers purchasing as principals and may also be sold to one or more purchasers directly, through applicable statutory exemptions, or through one or more agents designated from time to time, at amounts and prices and other terms determined by us or any selling shareholder. The securities may be sold from time to time in one or more transactions at fixed prices or not at fixed prices, such as market prices prevailing at the time of sale, prices related to such prevailing market prices or prices to be negotiated with purchasers, which prices may vary as between purchasers and during the period of distribution of the securities. The prospectus supplement relating to a particular offering of securities will identify each underwriter, dealer or agent engaged in connection with the offering and sale of such securities, the name or names of any selling shareholders, as well as the method of distribution and the terms of the offering of such securities, including the initial offering price (in the event the offering is a fixed price distribution), the manner of determining the offering price(s) (in the event the offering is not a fixed price distribution), the net proceeds to us and, to the extent applicable, any fees, discounts or any other compensation payable to underwriters, dealers or agents and any other material terms. See *"Plan of Distribution"*.

In connection with any offering of the securities other than an "at-the-market distribution" (as defined under applicable Canadian legislation), unless otherwise specified in the relevant prospectus supplement, the underwriters or agents may over-allot or effect transactions that stabilize or maintain the market price of the offered securities at a level above that which might otherwise prevail on the open market. Such transactions, if commenced, may be interrupted or discontinued at any time. See *"Plan of Distribution"*. No underwriter or dealer involved in an "at-the-market distribution" under this prospectus, no affiliate of such an underwriter or dealer and no person or company acting jointly or in concert with such underwriter or dealer will over-allot securities in connection with such distribution or effect any other transactions that are intended to stabilize or maintain the market price of the securities.

Our outstanding common shares are listed on the Toronto Stock Exchange, or the "TSX", and on NASDAQ Global Market, or "NASDAQ", under the symbol "BLU". On January 16, 2020, the last trading day prior to the date of this prospectus, the closing price of our common shares on the TSX and NASDAQ was Cdn$11.60 and US$8.60, respectively.

Our head office is located at 275 Armand-Frappier Boulevard, Laval, Quebec H7V 4A7, Canada.

Investors should be aware that the acquisition, holding or disposition of the securities described herein may have tax consequences both in the United States and in Canada. Such consequences for investors who are resident in, or citizens of, the United States and Canada may not be described fully herein. You should read the tax discussion contained in this prospectus and the applicable prospectus supplement with respect to a particular offering of the securities and consult your own tax advisor with respect to your own particular circumstances. No underwriter, agent or dealer has been involved in the preparation of this prospectus or performed any review of the contents of this prospectus.

Any investment in securities involves significant risks that should be carefully considered by prospective investors before purchasing securities. The risks outlined in this prospectus and in the documents incorporated by reference herein, including the applicable prospectus supplement, should be carefully reviewed and considered by prospective investors in connection with any investment in securities. See "*Risk Factors*".

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ABOUT THIS PROSPECTUS

We have not authorized anyone to provide any information other than that contained or incorporated by reference in this prospectus or any amendment or supplement to this prospectus. We do not take any responsibility for, or provide any assurance as to the reliability of, any other information that others may provide you. You should assume that the information appearing in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common shares, and that information appearing in any document incorporated by reference is accurate only as of the date of such document. Our business, financial condition, results of operations or prospects may have changed since those dates. This prospectus is not an offer to sell or the solicitation of an offer to buy our common shares in any circumstances under which such offer or solicitation is unlawful.

In this prospectus, unless the context otherwise permits, the terms "BELLUS Health", the "Company", "we", "us", and "our" refer to BELLUS Health Inc. and its subsidiaries, BELLUS Health Cough Inc. and BELLUS Health Corp. References to "Cdn$" and "$" are to Canadian dollars and "US$" are to U.S dollars.

All information permitted under applicable laws to be omitted from this prospectus will be contained in one or more prospectus supplements that will be delivered to purchasers together with this prospectus, unless an exemption from the prospectus delivery requirements has been granted or is otherwise available to us. Each prospectus supplement will be incorporated by reference in this prospectus for the purposes of securities legislation as of the date of the prospectus supplement and only for the purposes of the distribution of those securities to which the prospectus supplement pertains.

This prospectus includes market share information, epidemiology and industry data, pricing and commercial forecasts obtained from independent industry publications and surveys. References in such documents to research reports, surveys or articles should not be construed as depicting the complete findings of the entire referenced report, survey or article. The information in any such report, survey or article is not incorporated by reference in this prospectus. Although we believe these sources are reliable, we have not independently verified any of the data in such reports, surveys or articles. Some data is also based on our estimates, which are derived from our review of our internal surveys, as well as independent sources. We cannot and do not provide any assurance as to the accuracy or completeness of such information. Market forecasts, in particular, are likely to be inaccurate, especially over long periods of time.

FINANCIAL INFORMATION

Financial statements included or incorporated by reference herein have been prepared in accordance with IFRS as issued by the IASB and may not be comparable to financial statements of United States companies. Our financial statements are subject to audit in accordance with Canadian generally accepted auditing standards and/or the standards of the PCAOB and our auditor is subject to both Canadian auditor independence standards and the auditor independence standards of the PCAOB and the SEC.

ADDITIONAL INFORMATION

This prospectus is part of a registration statement on Form F-10 (the "**U.S. Registration Statement**") that the Company has or will file with the SEC under the United States Securities Act of 1933, as amended (the "**U.S. Securities Act**") relating to the common shares. Under the U.S. Registration Statement, the Company may, from time to time, sell common shares described in this prospectus in one or more offerings up to an aggregate offering amount of US$250,000,000. This prospectus, which forms a part of the U.S. Registration Statement, provides you with a general description of the common shares that the Company may offer and does not contain all of the information contained in the U.S. Registration Statement, certain items of which are contained in the exhibits to the U.S. Registration Statement, as permitted by the rules and regulations of the SEC. See "*Documents Filed as Part of the U.S. Registration Statement*". Statements included or incorporated by reference in this prospectus about the contents of any contract, agreement or other documents referred to are not necessarily complete, and in each instance, you should refer to the exhibits for a complete description of the matter involved. Each such statement is qualified in its entirety by such reference. Each time we sell securities under U.S. Registration Statement, we will provide a prospectus supplement that will contain specific information about the terms of that offering. The prospectus supplement may also add, update or change information contained in this prospectus. Before you invest, you should read both this prospectus and any applicable prospectus supplement together with additional information described under the heading "*Documents Incorporated by Reference*". **This prospectus does not contain all of the information set forth in the U.S. Registration Statement, certain parts of which are omitted in accordance with the rules and regulations of the SEC, or the schedules or exhibits that are part of the U.S. Registration Statement. Investors in the United States should refer to the U.S. Registration Statement and the exhibits thereto for further information with respect to the Company and the common shares.**

Our common shares are registered under Section 12(b) of the United States Securities Exchange Act of 1934, as amended (the "**U.S. Exchange Act**"), and accordingly, we are subject to the informational requirements of the U.S. Exchange Act and applicable Canadian requirements. In accordance with such requirements, we file reports and other information with the SEC and with securities regulatory authorities in Canada. Under the MJDS adopted by the United States and Canada, documents and other information that we file with the SEC may be prepared in accordance with the disclosure requirements of Canada, which are different from those of the United States. As a foreign private issuer, we are exempt from the rules the U.S. Exchange Act prescribing the furnishing and content of proxy statements, and our officers, directors and principal shareholders are exempt from the reporting and short-swing profit recovery provisions contained in Section 16 of the U.S. Exchange Act. Reports and other information filed by us with, or furnished to, the SEC may be accessed on the SEC's website at www.sec.gov. You may read and download any public document that we have filed with securities commission or similar regulatory authorities in Canada, on SEDAR at www.sedar.com.

DOCUMENTS INCORPORATED BY REFERENCE

Information has been incorporated by reference in this prospectus from documents filed with securities commissions or similar regulatory authorities in Canada. Copies of the documents incorporated by reference in this prospectus may be obtained upon request without charge from our Vice President, Finance at 275 Armand-Frappier Boulevard, Laval, Quebec H7V 4A7, Canada, telephone: (450) 680-4500, or by accessing our disclosure documents available through the Internet on SEDAR, which can be accessed at www.sedar.com. Some of the documents that we file with or furnish to the SEC are electronically available from the SEC's Electronic Document Gathering and Retreival System, commonly known as "EDGAR", and may be accessed at www.sec.gov. Our filings through SEDAR and EDGAR are not incorporated by reference in this prospectus except as specifically set forth herein.

Except to the extent that their contents are modified or superseded by a statement contained in this prospectus or in any other document that is also incorporated by reference in this prospectus, the following documents filed us with securities commissions or similar regulatory authorities in Canada are specifically incorporated by reference into, and form an integral part of, this prospectus:

(i) our annual information form dated March 13, 2019 for the fiscal year ended December 31, 2018;

(ii) our audited annual consolidated financial statements as at and for the years ended December 31, 2018 and 2017 together with the independent auditors' report thereon and our management's discussion and analysis dated February 20, 2019 in respect of those statements;

(iii) our management information circular dated March 13, 2019 in connection with our annual and special meeting of shareholders held on May 8, 2019;

(iv) our material change report dated August 20, 2019 regarding the consolidation of our common shares effected on August 15, 2019;

(v) our material change report dated August 27, 2019 regarding the appointment of Dr. Catherine Bonuccelli;

(vi) our material change report dated September 13, 2019 announcing the closing of our US$70 million public offering of common shares in Canada and the United States; and

(vii) our unaudited interim condensed consolidated financial statements for the three and nine-month periods ended September 30, 2019 (with the exception of the following notice included as the last sentence of the first paragraph of note 2(a) to these unaudited interim condensed financial statements: "These condensed consolidated interim financial statements have not been reviewed by the Company's auditors.") and our management's discussion and analysis dated November 13, 2019 in respect of those statements.

Any documents of the type described in Item 11.1 of Form 44-101F1 — *Short Form Prospectus Distributions* filed by us with the securities commissions or similar authorities in the provinces of Canada subsequent to the date of this prospectus and during the 25-month period that this prospectus, including any amendments hereto, remains valid shall be deemed to be incorporated by reference in this prospectus. Documents referenced in any of the documents incorporated by reference in this prospectus but not expressly incorporated by reference therein or herein and not otherwise required to be incorporated by reference therein or herein are not incorporated by reference in this prospectus.

Notwithstanding anything herein to the contrary, any statement contained in a document incorporated or deemed to be incorporated by reference herein will be deemed to be modified or superseded for the purposes of this prospectus, to the extent that a statement contained herein or in any other subsequently filed document that also is or is deemed to be incorporated by reference in this prospectus modifies or supersedes such statement. Any statement so modified or superseded shall not constitute a part of this prospectus, except as so modified or superseded. The modifying or superseding statement need not state that it has modified or superseded a prior statement or include any other information set forth in the document that it modifies or supersedes. Making such a modifying or superseding statement shall not be deemed an admission for any purposes that the modified or superseded statement, when made, constituted a misrepresentation, an untrue statement of a material fact or an omission to state a material fact that is required to be stated or that is necessary to make a statement not misleading in light of the circumstances in

which it was made. Without limiting the generality of the foregoing, the description of our business appearing in this prospectus under the heading "Business of the Company" modifies and supersedes, to the extent inconsistent therewith, the description of our business contained under the heading "Business" in our annual information form dated March 13, 2019; the regulatory disclosure appearing in this prospectus under the heading "Regulatory Matters" modifies and supersedes, to the extent inconsistent therewith, the regulatory disclosure contained under the heading "Business" in our annual information form dated March 13, 2019; the risk factors appearing in this prospectus under the heading "Risk Factors" modifies and supersedes, to the extent inconsistent therewith, the risk factors contained under the heading "Risk Factors" in our annual information form dated March 13, 2019.

Upon a new annual information form and annual consolidated financial statements being filed by us with the applicable Canadian securities commissions or similar regulatory authorities in Canada during the period that this prospectus is effective, the previous annual information form, the previous annual consolidated financial statements and all interim consolidated financial statements and in each case the accompanying management's discussion and analysis, and material change reports, filed prior to the commencement of the financial year of the Company in which the new annual information form is filed shall be deemed to no longer be incorporated into this prospectus for purpose of future offers and sales of securities under this prospectus. Upon interim consolidated financial statements and the accompanying management's discussion and analysis being filed by us with the applicable Canadian securities commissions or similar regulatory authorities during the period that this prospectus is effective, all interim consolidated financial statements and the accompanying management's discussion and analysis filed prior to such new interim consolidated financial statements and management's discussion and analysis shall be deemed to no longer be incorporated into this prospectus for purposes of future offers and sales of Securities under this prospectus. In addition, upon a new management information circular for an annual meeting of shareholders being filed by us with the applicable Canadian securities commissions or similar regulatory authorities during the period that this prospectus is effective, the previous management information circular filed in respect of the prior annual meeting of shareholders shall no longer be deemed to be incorporated into this prospectus for purposes of future offers and sales of securities under this prospectus.

To the extent that any document or information incorporated by reference into this prospectus is included in any report on Form 6-K, Form 40-F or Form 20-F (or any respective successor form) that is filed with or furnished to the SEC after the date of this prospectus, such document or information shall be deemed to be incorporated by reference as an exhibit to the U.S. Registration Statement of which this prospectus forms a part. In addition, we may incorporate by reference into this prospectus, or the U.S. Registration Statement of which it forms a part, other information from documents that we will file with or furnish to the SEC pursuant to Section 13(a) or 15(d) of the U.S. Exchange Act, if and to the extent expressly provided therein.

A prospectus supplement containing the specific variable terms in respect of an offering of the common shares will be delivered to purchasers of such common shares together with this prospectus, unless an exemption from the prospectus delivery requirements has been granted or is otherwise available, and will be deemed to be incorporated by reference into this prospectus as of the date of such prospectus supplement only for the purposes of the offering of the securities covered by such prospectus supplement.

DOCUMENTS FILED AS PART OF THE U.S. REGISTRATION STATEMENT

The following documents have been, or will be, filed with the SEC as part of the U.S. Registration Statement of which this prospectus is a part insofar as required by the SEC's Form F-10:

- the documents listed under "Documents Incorporated by Reference" in this prospectus;
- the consent of KPMG LLP, the Company's independent auditor;
- the consent of Davies Ward Phillips & Vineberg LLP, the Company's Canadian counsel; and
- powers of attorney of the Company's directors and officers, as applicable.

FORWARD-LOOKING STATEMENTS

Certain statements contained in this prospectus, any prospectus supplement and the documents incorporated by reference herein and therein may constitute "forward-looking information" within the meaning of applicable securities laws in Canada and "forward-looking statements" within the meaning of the United States Private Securities Litigation Reform Act of 1995, as amended (collectively, "forward-looking statements"), which involve known and unknown risks, uncertainties and other factors which may cause the actual results, performance or achievements of the Company, or industry results, to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. These forward-looking statements include information about possible or assumed future results of our business, financial condition, results of operations, liquidity, objectives and strategies to achieve those objectives, as well as statements with respect to our beliefs, targets, expectations, anticipations, estimates or intentions. In some cases, you can identify forward-looking statements by terminology such as "believe", "may", "estimate", "continue", "anticipate", "intend", "should", "plan", "expect", "predict", "potential", "could", "assume", "project", "guidance" or the negative of these terms or other similar expressions, although not all forward-looking statements include such words. The statements we make regarding the following matters are forward-looking by their nature and are based on certain of the assumptions noted below:

- our aim to develop and commercialize BLU-5937 for the treatment of hypersensitization disorders, including chronic cough and chronic pruritus;
- our aim to complete additional preclinical studies on BLU-5937;
- our aim to pursue the Phase 2 clinical trial on BLU-5937 for the treatment of patients with refractory chronic cough in 2019 with topline data in mid-2020, and initiate later stage clinical studies thereafter;
- our aim to initiate a Phase 2 clinical trial on BLU-5937 for the treatment of patients with chronic pruritus associated with atopic dermatitis, in Q2 2020, with topline data expected in mid-2021;
- our aim to further explore the potential of BLU-5937 for the treatment of other afferent hypersensitization-related conditions;
- our expectations relating to the timing and cost of significant preclinical study and clinical trial milestones;
- our expectations with respect to the timing and cost of the research and development activities of BLU-5937;
- the function, potential benefits, effectiveness and safety of our product candidates, including BLU-5937;
- our expectations with respect to pre-commercialization activities related to the commercial launch of BLU-5937;
- our estimates and assessment of the potential markets for our product candidates;
- our expectations regarding pricing and acceptance of our product candidates by the market;
- the benefits and risks of our product candidates as compared to others;
- our aim to obtain regulatory approvals to market our product candidates;
- our expectations with respect to the cost of preclinical studies and clinical trials and commercialization of our product candidates, including BLU-5937;
- our current and future capital requirements and anticipated sources of financing or revenue;
- our expectations regarding the protection of our intellectual property;
- our business strategy;
- potential milestone payments and royalties pursuant to license agreements and other partnerships; and

- our development and partnership plans and objectives.

The preceding list is not intended to be an exhaustive list of all of our forward-looking statements.

Conclusions, forecasts and projections set out in forward-looking information are based on our current objectives and strategies and on expectations and estimates and other factors and assumptions that we believe to be reasonable at the time applied but may prove to be incorrect. These include, but are not limited to:

- the function, potential benefits, effectiveness and safety of BLU-5937;
- the benefits and risks of our product candidates as compared to others;
- progress, timing and costs related to the development, completion and potential commercialization of our product candidate;
- estimates and projections regarding our industry;
- market acceptance of our product candidate;
- future success of current research and development activities;
- achievement of development and commercial milestones, including forecasted preclinical study and clinical trial milestones;
- our reliance on third parties to conduct preclinical studies and clinical trials for BLU-5937;
- that the timeline and costs for our preclinical and clinical programs are not incorrectly estimated or affected by unforeseen circumstances;
- absence of material deterioration in general business and economic conditions;
- the receipt of regulatory and governmental approvals for research and development projects and timing thereof;
- the availability of tax credits and financing for research and development projects, and the availability of financing on favorable terms;
- the accuracy of our estimates regarding future financing and capital requirements and expenditures;
- the achievement of our forecasted cash burn rate;
- the sufficiency and validity of our intellectual property rights;
- our ability to secure, maintain and protect our intellectual property rights, and to operate without infringing on the proprietary rights of others or having third parties circumvent the rights owned or licensed by us;
- our ability to source and maintain licenses from third-party owners on acceptable terms and conditions;
- absence of significant changes in Canadian dollar-U.S. dollar and other foreign exchange rates or significant variability in interest rates;
- the absence of material changes in market competition;
- our ability to attract and retain skilled staff;
- our ability to maintain ongoing relations with employees and business partners, suppliers and other third parties;
- the accuracy of the market research, third-party industry data and forecasts relied upon by us; and
- the absence of adverse changes in relevant laws or regulations.

There are important factors that could cause our actual results, levels of activity, performance or achievements to differ materially from the results, levels of activity, performance or achievements expressed or implied by the forward-looking statements. See "*Risk Factors*" in this prospectus. Should one or more of the risks, uncertainties or other factors outlined in this prospectus materialize, our objectives, strategies or intentions change, or any of the factors or assumptions underlying the forward-looking information prove incorrect, our actual results and our plans and targets could vary significantly from what we currently foresee. Accordingly, we warn investors to exercise caution when considering statements containing forward-looking information and that it would be unreasonable to rely on such statements as creating legal rights regarding our future results or plans or targets. All of the forward-looking information in this prospectus is qualified by the cautionary statements herein.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

Before making any investment decision in respect of the securities and for a detailed discussion of the risks and uncertainties associated with our business, its operations and its financial targets, performance and condition and the material factors and assumptions underlying the forward-looking information herein and therein, fully review the disclosure incorporated by reference in and included in this prospectus and any prospectus supplement, including the risks described in the "Risk Factors" section of this prospectus.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that future results, levels of activity, performance and events and circumstances reflected in the forward-looking statements will be achieved or will occur. Forward-looking statements made in a document incorporated by reference in this prospectus are made as at the date of the original document and have not been updated by us except as expressly provided for in this prospectus. Except as required by law, we undertake no obligation to update publicly any forward-looking statements for any reason after the date of this prospectus, to conform these statements to actual results or to changes in our expectations.

THE COMPANY

The company was incorporated on April 12, 2012 under the *Canada Business Corporations Act* and is the successor of BELLUS Health Inc., a company incorporated on June 17, 1993 (known as Neurochem Inc. prior to April 15, 2008). We have two wholly-owned subsidiaries, BELLUS Health Cough Inc., also incorporated under the *Canada Business Corporations Act*, and BELLUS Health Corp., incorporated under the laws of the state of Delaware. Our head office is located at 275 Armand-Frappier Boulevard, Laval, Quebec H7V 4A7, Canada.

Our outstanding common shares are listed on the TSX and NASDAQ under the symbol "BLU".

Our website address is www.bellushealth.com. Information contained on, or that can be accessed through, our website does not constitute a part of this prospectus and is not incorporated by reference herein. We have included our website address in this prospectus solely for informational purposes. Our agent for service of process in the United States is CT Corporation System and its telephone number is (202) 572-3111.

RECENT DEVELOPMENTS

There have been no material developments in the business of the Company, since the date of our most recent interim financial statements, which have not been disclosed in this prospectus.

BUSINESS OF THE COMPANY

Overview

We are a clinical stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of chronic cough and other hypersensitization disorders. Our product candidate,

BLU-5937, is a twice daily oral small molecule specifically designed to be a highly selective inhibitor of the P2X3 receptor, a clinically validated target linked to hypersensitivity. We are developing BLU-5937 for the treatment of chronic cough and chronic pruritus, or chronic itch. These hypersensitization-related disorders, which share a common pathophysiology that is mediated through the P2X3 receptor, represent areas of significant unmet medical need and potentially large market opportunities. In July 2019, we enrolled our first patient in our ongoing Phase 2 clinical trial for BLU-5937 for the treatment of refractory chronic cough, with topline data expected in mid-2020. We also expect to initiate a Phase 2 clinical trial of BLU-5937 for the treatment of chronic pruritus associated with atopic dermatitis, also known as eczema, in Q2 2020, with topline data expected in mid-2021. We have exclusive worldwide development and commercialization rights to BLU-5937 in all indications.

We believe that BLU-5937 has best-in-class selectivity for the homotrimeric P2X3 receptor, or "P2X3". Given this selectivity, we believe that BLU-5937 has the potential to significantly alleviate refractory chronic cough and chronic pruritus symptoms while limiting or potentially eliminating the taste loss and taste alteration observed with the most advanced P2X3 receptor inhibitor in development, Merck & Co.'s gefapixant, which has low selectivity for P2X3. While we are initially focused on development in chronic cough and chronic pruritus, we are also evaluating the potential role of P2X3 inhibition in the treatment of other afferent hypersensitization-related disorders.

Chronic cough, our lead indication for BLU-5937, is a cough lasting more than eight weeks, and may have a significant adverse impact on patients' quality of life. It is estimated that more than 26 million adults in the United States suffer from chronic cough, with more than 2.6 million of those people having refractory chronic cough lasting more than one year. Many patients report that their condition has a marked effect on their quality of life including sleep disruption, tiredness, incontinence, and disrupting social interactions. Currently, there is no therapy approved specifically for the treatment of refractory chronic cough. Available treatment options are limited and may have inadequate benefit and/or significant safety and tolerability issues, including significant taste alteration or loss. We believe that BLU-5937, if approved, may be adopted by physicians as an oral cough therapy either as an adjunct to treatments targeting the underlying cause of the chronic cough or as a monotherapy in patients for whom cough is the primary etiology.

In November 2018, we reported positive results from our Phase 1 clinical trial in 90 healthy volunteers, in which we observed that BLU-5937 had a favorable tolerability and safety profile at all doses tested. At doses of 50 mg to 100 mg, there was only one subject out of 24 (<5%) who reported taste alteration, which was transient, sporadic and only occurred on the first day of dosing. None of the 24 subjects (0%) reported any taste loss. We believe that doses of 50 mg to 100 mg administered twice-daily (BID) would result in the desired level of therapeutic activity. In contrast, gefapixant was reported to cause taste alteration and/or taste loss in up to 80% of patients at the therapeutically relevant dose of 50 mg BID in a Phase 2 clinical trial. We are currently conducting a Phase 2 clinical trial of BLU-5937 in patients with refractory chronic cough. This randomized, double-blind, placebo-controlled, dose-escalation, two-period crossover trial is designed to assess the efficacy, safety, and tolerability of BLU-5937 at four doses; 25, 50, 100 and 200 mg, administered orally, BID. Doses are escalated at four-day intervals. We expect that approximately 65 patients with refractory chronic cough will be enrolled at approximately fifteen clinical sites in the United Kingdom and the United States. We enrolled our first patient in July 2019, are actively recruiting patients and expect to report topline data in mid-2020.

Chronic pruritus, commonly known as chronic itch, our second indication for BLU-5937, is characterized as an ongoing, uncomfortable, irritating sensation that makes a person want to scratch, persists for more than six weeks and may have a significant adverse impact on patients' quality of life. Atopic dermatitis, also known as eczema, is a non-contagious itchy skin disorder characterized by the presence of dry and scaly patches on the skin of the scalp, forehead, arms, torso and face, particularly the cheeks. The itch associated with atopic dermatitis can be so intense that repeated scratching can lead to skin lesions, bleeding and infection. It is estimated that 16.9 million adults in the United States are affected by atopic dermatitis, with pruritus being the primary complaint among such patients. Of the total population of adults affected by atopic dermatitis in the United States, it is estimated that three million of those are diagnosed with the disease, and of those diagnosed, it is estimated that 2.25 million patients are actively

being treated by a physician. Accordingly, we believe that there is a significant market opportunity for a therapy for chronic pruritus associated with atopic dermatitis. Despite currently available treatments, an estimated 40-50% of atopic dermatitis patients report having inadequate relief of their pruritus and are in need of new, efficacious pruritus therapies.

We plan to initiate a randomized, double-blind, placebo-controlled, parallel group design Phase 2 clinical trial to assess the efficacy, safety, and tolerability of BLU-5937 in approximately 100 patients suffering from moderate to severe chronic pruritus associated with mild to moderate atopic dermatitis. The trial is expected to be a two-arm study comparing BLU-5937 to placebo, each administered orally, twice-daily (BID), for four weeks. We expect to initiate the trial in Q2 2020 and report topline data in mid-2021.

We have exclusive worldwide development and commercialization rights to BLU-5937 in all indications. Our BLU-5937 program is protected by a comprehensive patent estate comprised of issued and pending patents. We have secured composition of matter patent coverage for BLU-5937 in all major pharmaceutical markets: the United States of America, Europe, Japan and China until 2034. Under certain circumstances, such patent term may be extended for up to five years in certain jurisdictions such as the United States, Europe and Japan. In addition, we have secured methods of use patent coverage in the United States for avoiding loss of taste response while treating a chronic cough patient, through treatment with BLU-5937, expiring in 2038.

We are led by a team of executives with extensive experience in drug development, having held leadership roles at numerous biopharmaceutical companies, including Astra Zeneca, Biochem Pharma and GlaxoSmithKline. Our chronic cough clinical advisory board comprises experts who have acted as lead investigators in numerous chronic cough clinical trials, including those conducted with gefapixant. Since 2017, we have completed several financings with specialized U.S.-based healthcare investing firms.

In August 2019, we appointed Dr. Catherine Bonuccelli, MD to the role of Chief Medical Officer. Dr. Bonuccelli is a pediatric pulmonologist who brings over 20 years of pharmaceutical experience and significant expertise in clinical development and commercialization of respiratory and non-respiratory products. Prior to joining BELLUS Health, Dr. Bonuccelli held a number of leadership positions focusing on late-stage clinical development of large and small molecule programs in respiratory and inflammation therapeutic areas.

Our Pipeline

The following table sets forth the status and initial focus of BLU-5937.

PROGRAM	DEVELOPMENT				STATUS	
Indication	Preclinical	Phase 1	Phase 2	Phase 3	Worldwide Rights	Next Anticipated Milestone
BLU-5937						
Refractory Chronic Cough					Bellus HEALTH	Mid-2020: Topline data
Chronic Pruritus Associated with Atopic Dermatitis					Bellus HEALTH	Q2 2020: Phase 2 initiation

Our Strategy

We are focused on the development and commercialization of BLU-5937 as a potential differentiated treatment option for chronic cough patients, as well as for the treatment of chronic pruritus associated with atopic dermatitis and other hypersensitization-related disorders. The key elements of our strategy are:

- *Advance the development of BLU-5937 in the treatment of chronic cough, our lead indication.* We are focused on efficiently developing BLU-5937 to treat patients with chronic cough. We are actively recruiting patients in a Phase 2 clinical trial to evaluate the efficacy, safety, and tolerability

of BLU-5937 in refractory chronic cough patients at four doses: 25 mg, 50 mg, 100 mg and 200 mg BID. We expect topline data in mid-2020. If our Phase 2 clinical trial is successful, we expect to initiate either a Phase 2b or a Phase 2/3 trial to further pursue the development of BLU-5937 for the treatment of chronic cough.

- *Advance the development of BLU-5937 in the treatment of chronic pruritus.* We expect to initiate a Phase 2 clinical trial in Q2 2020 to evaluate the efficacy, safety and tolerability of BLU-5937 in chronic pruritus associated with atopic dermatitis, a hypersensitization-related disorder, with topline data expected in mid-2021.

- *Maximize the value of BLU-5937 by maintaining flexibility to develop and commercialize our product independently or through collaborations.* We have exclusive worldwide development and commercialization rights for BLU-5937 in all indications. We may choose to pursue the development and commercialization of BLU-5937 independently or through collaborations with third parties.

- *Leverage our proprietary P2X3 antagonist technology platform to pursue other hypersensitization-related conditions.* We are evaluating the potential role of P2X3 inhibition in the treatment of other afferent hypersensitization-related disorders.

Chronic Cough

A Highly Prevalent Condition

Coughing is a reflex mechanism and the body's way of clearing irritants or mucus from the airways and can be either acute or chronic in nature. Chronic cough is classified as a cough lasting for more than eight weeks, and is usually associated with an underlying respiratory condition, such as asthma or chronic obstructive pulmonary disease ("**COPD**"), but can also be caused by other common non-respiratory conditions (e.g. allergic rhinitis or gastroesophageal reflux) or certain medications (e.g. ACE inhibitors). Notably, many cases of refractory chronic cough have no identifiable cause, a condition often referred to as unexplained chronic cough.

Chronic cough occurs when the nerves involved in the cough response become hypersensitive. For example, the coughing that occurs from a bad cold can sensitize the nerves involved in the cough response. The cough reflex can then become extremely sensitive to the point where coughing itself triggers more coughing. This can continue for an extended period, even after the trigger, such as the cold, has resolved.

Chronic cough can have a significant impact on quality of life, including debilitating physical and psychosocial burden. Fatigue, sleep disturbance, vomiting, chest pains, and incontinence can occur, and patients with chronic cough often experience social embarrassment. A study found that more than half of all chronic cough patients suffer from clinical depression.

In the United States, it is estimated that more than 26 million adults, representing approximately 10% of the adult population, suffer from chronic cough, of which more than 2.6 million have refractory chronic cough lasting for more than one year. These estimates are based on a market assessment commissioned by us in October 2018 and conducted by Bluestar BioAdvisors LLC (formerly known as Torreya Insights LLC) through an evaluation of chronic cough epidemiology and pricing estimates.

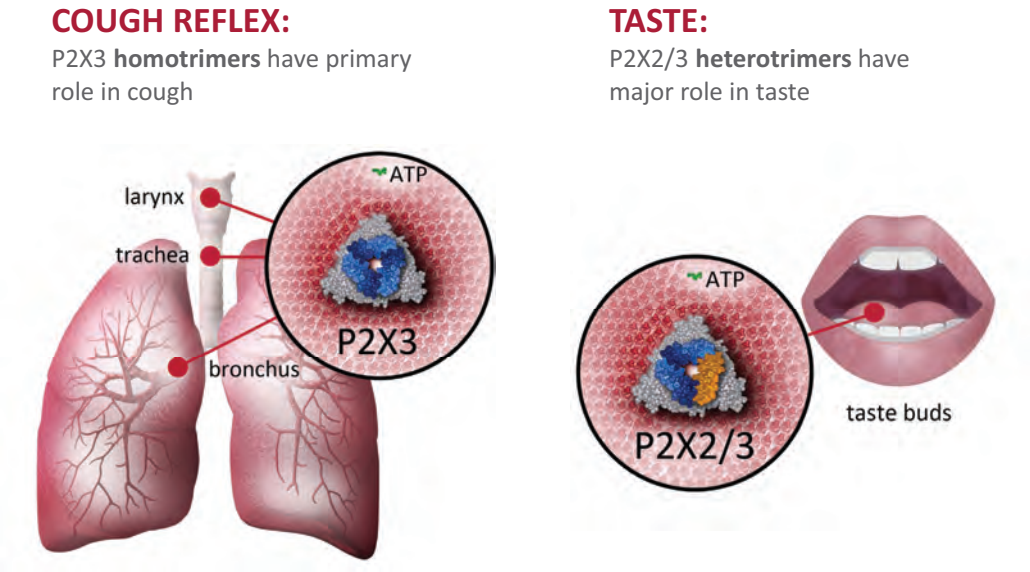
Limitations of Current Refractory Chronic Cough Therapies

Refractory chronic cough represents a substantial unmet medical need and current treatment options have demonstrated limited efficacy and/or have safety/tolerability issues. We believe the FDA has not approved an antitussive agent in over 60 years, the last antitussive agent it approved being dextromethorphan in 1958. Commonly used cough drugs, such as those incorporating dextromethorphan as their primary active ingredient, offer limited benefit, if any, to chronic cough patients. Benzonatate anesthetizes the stretch receptors in the lungs, but offers only temporary relief and may cause serious side effects if the capsule is crushed. Off-label treatment options, such as gabapentin and pregabalin, have shown variable efficacy and significant central nervous system side effects. The use of opioids, such as low-dose morphine and codeine, have shown some efficacy, but their use is controversial due to the potential for

addiction and other serious side effects such as drowsiness, nausea, constipation, respiratory depression and potential for addiction. Speech therapy has shown some efficacy, especially in combination with pharmacotherapy. Nevertheless, such therapy generally requires patient referral to specialized cough clinics with highly-trained medical personnel and a significant effort and time commitment by the patient.

Selective P2X3 Receptor Inhibition: A Promising and Clinically Validated Therapeutic Approach in Chronic Cough

The only clinically validated treatments in development for refractory chronic cough are molecules that inhibit the P2X3 receptor. P2X3 receptors are ATP-gated ion channels that belong to a family of purinergic receptors. Members of this family assemble as homotrimeric (three subunits of P2X3) or heterotrimeric (two subunits of P2X3 and one subunit of P2X2 (i.e., P2X2/3)) ion channels and are widely expressed in non-excitatory and excitatory cells, such as afferent neurons. Afferent sensory neurons are the primary conduit for sensory information and the primary site that may undergo modulation leading to persistently altered sensation, including hypersensitivity. ATP, acting via P2X3 receptors, is believed to be a key mediator of these changes. The ability to inhibit the binding of ATP to the P2X3 receptor has been shown to be a promising path in the search for therapeutics to treat disorders driven by neuronal hypersensitivity. ATP signaling via these P2X receptors is also necessary for successful transmission of information from taste cells to the sensory neurons that innervate the taste buds. In preclinical studies of double-knock out mice lacking both P2X2 and P2X3 purinoceptors, abolition of taste sensation was observed, whereas single knock-out of either the P2X2 or P2X3 receptor causes only moderate taste disturbance. We, therefore, believe that selective P2X3 inhibitors, such as BLU-5937, have the potential to mediate aberrant ATP signaling in conditions like chronic cough, chronic pruritus and other hypersensitization disorders, while limiting or potentially eliminating taste loss and taste alteration observed with gefapixant, a less selective P2X3 inhibitor that also inhibits the P2X2/3 receptor.



Gefapixant is the most advanced P2X3 receptor inhibitor in clinical development and is currently undergoing clinical evaluation in two Phase 3 studies. Gefapixant is a non-narcotic, low selectivity P2X3 inhibitor which has been shown to alleviate refractory chronic cough symptoms and improve patients' quality of life in Phase 2 clinical studies.

Results from an initial Phase 2, double-blind clinical trial in patients with refractory chronic cough showed that treatment with a high dose of gefapixant (600 mg BID) led to a significant reduction in mean daytime cough frequency compared with placebo. A subsequent dose-escalation trial confirmed the clinical activity of gefapixant in refractory chronic cough patients even when testing a much lower dose (50 mg BID). Results of a randomized, double-blind, 12-week, placebo-controlled trial also showed significant and clinically meaningful reductions in awake cough frequency and 24-hour cough frequency after treatment with gefapixant. Across all Phase 2 trials, dose-dependent taste alteration and taste loss was the most

commonly reported adverse event. In a Phase 2b trial in which 50 mg gefapixant was given twice daily, 81% of patients reported taste side effects, 48% of patients reported taste alteration, 24% had partial loss of taste and 21% had complete taste loss. Of those patients who reported taste disturbances, approximately 40% rated them as "very" or "extremely bothersome". Ten percent of participants discontinued participation in the trial prematurely due to the taste disturbance and/or taste loss. These side effects were reported to persist for the duration of the trial but were reported to subside once treatment ceased.

BLU-5937, Our Highly Selective P2X3 Inhibitor Product Candidate

We are developing BLU-5937, a potent, highly selective, orally bioavailable small molecule inhibitor of the P2X3 receptor, as an oral therapy to reduce cough frequency in chronic cough patients. Advances in the understanding of possible mechanisms underlying chronic cough have paved the way for product candidates targeting the P2X3 receptors, such as BLU-5937. To date, several clinical studies have validated the potential of targeting this receptor and ongoing clinical studies seek to further evaluate the efficacy and safety of P2X3-targeting agents in refractory chronic cough. We believe BLU-5937's characteristics shown in preclinical studies and a Phase 1 trial position it as a differentiated treatment option in the P2X3 inhibitors class. These include:

BLU-5937 is a potent inhibitor of P2X3 that has the potential to significantly alleviate refractory chronic cough symptoms

The high potency and selectivity of BLU-5937 for P2X3 receptors was shown in vitro by inhibiting ATP-evoked P2X3 receptor activity in cloned human P2X3 channels expressed in mammalian cells. The concentration of BLU-5937 needed to inhibit 50% of the P2X3 activity (IC50) in this assay was established at 25 nM, which was approximately three times more potent than gefapixant.

In vitro, BLU-5937 was observed to block ATP-induced sensitization and firing activity of primary nociceptors in rat dorsal root ganglions through P2X3 receptor inhibition.

In the guinea pig cough model, we observed that BLU-5937 significantly reduced, in a dose-dependent fashion, the histamine or ATP-induced enhancement in number of citric acid-induced coughs. In these validated models of cough, the antitussive effect of BLU-5937 was observed to be comparable to that of gefapixant.

BLU-5937 is highly selective for P2X3 that has the potential to significantly reduce or eliminate taste side effects

We believe that BLU-5937, which has been specifically designed to be a highly selective inhibitor of the P2X3 receptor, has the potential to significantly alleviate refractory chronic cough while maintaining taste function. The high selectivity of BLU-5937 for P2X3 receptors was observed in vitro by inhibiting receptor activity in cloned human P2X3 and P2X2/3 channels expressed in mammalian cells. The BLU-5937 selectivity ratio was observed to be, on average, greater than 1,500 times in favor of P2X3 as compared to P2X2/3, whereas the selectivity ratio for gefapixant was observed to be approximately three to seven fold higher for P2X3 as compared to P2X2/3.

In a rat behavioral taste model, we observed that BLU-5937 did not alter taste perception compared to control animals, whereas gefapixant had a significant inhibitory effect on taste (>80% of mice experienced taste alteration or loss). We believe that the lack of effect of BLU-5937 on taste perception, even at high doses, is due to its higher selectivity for the P2X3 versus P2X2/3 receptors on the taste buds.

In a Phase 1 trial with healthy volunteers given BLU-5937, at the anticipated therapeutic doses of 50 mg to 100 mg, no subjects reported loss of taste perception and only one subject out of 24 (<5%) reported a transient and sporadic taste alteration, which occurred only on the first day of dosing.

BLU-5937 is orally bioavailable and has a half-life that supports dosing as a tablet twice daily

The safety, tolerability and pharmacokinetic profile of BLU-5937 was assessed in preclinical studies in which we observed that BLU-5937 exhibited good oral bioavailability, low predicted clearance in humans, no blood-brain barrier permeability and a favorable tolerability profile.

The Phase 1 data demonstrated a favorable pharmacokinetic profile for BLU-5937: rapid absorption with maximum plasma concentration achieved within one to two hours post-dose, dose-proportionally plasma concentration increases and a plasma half-life of four to nine hours that supports a twice a day dosing schedule.

The pharmacokinetic profile from the Phase 1 trial also supported that the drug can be taken without regard to meals, which is convenient for patients and supports compliance. In addition, there was no evidence of significant drug accumulation upon repeated dose administration. Based on achieving targeted receptor inhibition and activity in preclinical studies and on achieving comparative drug blood levels of a clinically validated comparator, after correcting for pharmacokinetic and potency differences, we anticipate that drug levels required for optimal inhibition of cough will be achieved at 50 mg to 100 mg BID.

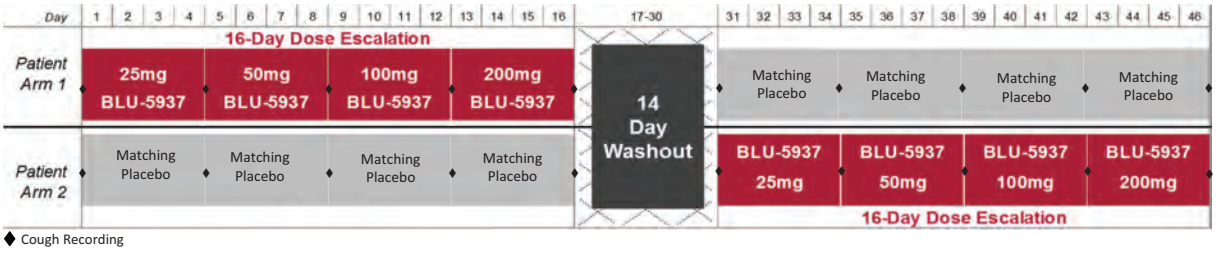
We believe that BLU-5937, if approved, may be adopted by physicians as an oral cough therapy either as an adjunct to treatments targeting the underlying cause of the chronic cough or as a monotherapy in patients for whom the cough is the primary etiology.

BLU-5937 Ongoing Phase 2 Clinical Trial in Refractory Chronic Cough

We are currently conducting a Phase 2 clinical trial of BLU-5937 patients with refractory chronic cough, which we refer to as the RELIEF (A Randomized, Double-blind, Placebo-Controlled, Crossover, Dose Escalation Study of BLU-5937 in Subjects with Refractory Chronic Cough) trial. The trial was initiated in July 2019 and we expect to report topline data in mid-2020.

The RELIEF trial is a randomized, double-bind, placebo-controlled, dose escalation and two-period crossover design trial to assess the efficacy, safety and tolerability of BLU-5937 at four doses: 25, 50, 100 and 200 mg BID. Doses are escalated at four-day intervals. In the Phase 1 trial, 2.5% of subjects tested at these doses reported a taste alteration event. Approximately 65 patients with refractory chronic cough are expected to be enrolled at approximately 15 clinical sites located in the United Kingdom and United States. We enrolled the first patient in the RELIEF trial at the end of July 2019 and are actively recruiting patients.

The four doses selected for the RELIEF trial were based on pharmacokinetic/pharmacodynamic modeling using data gathered from preclinical cough studies, data from a Phase 2 clinical trial with a class competitor and the BLU-5937 Phase 1 trial. Based on that modeling, it is anticipated that the optimal therapeutic doses will be 50 mg to 100 mg BID, however, to allow a better characterization of the dose response range and proper dose selection for future clinical studies, the 25 mg BID and 200 mg BID doses are also being evaluated.



The primary efficacy endpoint of the RELIEF trial is the change from baseline in awake cough frequency as measured by a cough recorder at the end of each dose level. Secondary efficacy endpoints include the change in 24-hour cough frequency and the change in the Leicester Cough Questionnaire, Cough Severity Visual Analogue Scale (VAS) and the Global Rating of Change Scale.

The key inclusion criteria in the RELIEF trial are that patients must have refractory chronic cough for at least one year, an awake cough count of $\geq$ 10 per hour (Awake Cough Count at Screening) and a score of $\geq$ 40mm on the Cough Severity VAS at Screening. Current or past smoking (within the past six months) and a diagnosis of chronic obstructive pulmonary disease, bronchiectasis, or idiopathic pulmonary fibrosis are key exclusion criteria.

We will also collect taste adverse event data as part of the RELIEF trial. Phase 1 results showed that at the anticipated therapeutic doses of 50 mg to 100 mg BID, no subjects administered BLU-5937 reported any loss of taste perception and only one subject out of 24 (<5%) reported transient and sporadic taste

alteration only on the first day of dosing. No subject reported total loss of taste at any dose. To fully characterize any potential taste disturbance effects seen in the RELIEF trial, a questionnaire will be provided to patients who report taste side effects in the trial.

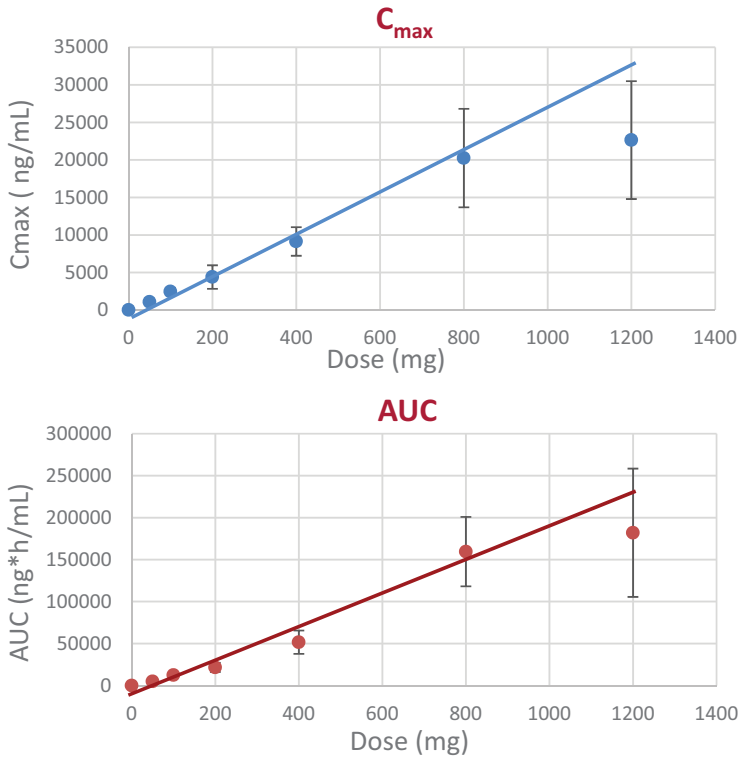
The RELIEF trial is being conducted with Illingworth Research Group, a clinical research organization which has conducted multiple clinical trials in chronic cough. Each of the trial sites are experienced in conducting chronic cough trials. Many of the sites are Centers of Excellence for the treatment of chronic cough and have access to a significant pool of patients.

BLU-5937 Phase 1 Trial

Trial Data

In November 2018, we completed a Phase 1 trial for BLU-5937 in 90 healthy adult volunteers, in which we observed that BLU-5937 is well tolerated, with a favorable pharmacokinetic profile. BLU-5937 was observed to be rapidly absorbed, achieving maximum plasma concentration within one to two hours. Plasma half-life was established at four to nine hours, supporting BID dosing. Based on preclinical efficacy studies and comparison with drug levels achieved with a clinically validated comparator, after correcting for pharmacokinetic and potency differences, we anticipate that drug levels required for optimal inhibition of cough will be achieved at 50 mg to 100 mg BID. As shown in the graphs below, we observed that BLU-5937 plasma concentration (Cmax and AUC) increased dose-proportionally and was not affected by food, supporting BLU-5937 administration without regard to meals.

Phase I Pharmacokinetic Profile and Dosing



The overall incidence of adverse events was comparable between placebo (50%) and BLU-5937 (44%). At the anticipated therapeutic doses of 50 mg to 100 mg, no subjects administered BLU-5937 reported any loss of taste perception and only one subject out of 24 (<5%) reported transient and sporadic taste alteration. No subject reported total loss of taste at any dose levels. This taste effect was reported only on the first day out of seven days of dosing by a subject receiving 100 mg BID. No subject out of 16 reported any taste loss or taste alteration at 200 mg.

Incidence of Most Frequent Adverse Events (>5% Incidence) in All Cohorts (SAD + MAD)

AEs N (%)	Placebo (n=18)	50mg (n=8)	100mg (n=16)	200mg (n=16)	400mg (n=16)	800mg (n=8)	1200mg (n=8)	Total BLU-5937 (n=72)
Taste Alteration	0 (0%)	0 (0%)	1 (6%)	0 (0%)	6 (38%)	5 (63%)	2 (25%)	14 (19%)
Headache	1 (6%)	0 (0%)	2 (13%)	1 (6%)	1 (6%)	2 (25%)	2 (25%)	8 (11%)
Hypoaesthesia	0 (0%)	0 (0%)	0 (0%)	3 (19%)	2 (13%)	3 (38%)	0 (0%)	8 (11%)
Dizziness	0 (0%)	0 (0%)	0 (0%)	0 (0%)	2 (13%)	1 (13%)	1 (13%)	4 (6%)
Nausea	1 (6%)	0 (0%)	0 (0%)	1 (6%)	1 (6%)	2 (25%)	2 (25%)	6 (8%)
Dyspepsia	0 (0%)	0 (0%)	1 (6%)	0 (0%)	2 (13%)	1 (13%)	0 (0%)	4 (6%)

At supra-therapeutic doses (200 mg to 1200 mg), two subjects out of 48 (4%) reported transient and sporadic partial loss of taste, and 13 subjects out of 48 (27%) reported transient and sporadic taste alteration. All taste-related events were transitory and sporadic in nature; one was rated moderate and all others were rated mild. The other most frequent adverse events reported in the Phase 1 trial (>5%) were: headache (11%), hypoaesthesia (11%), nausea (8%), dizziness (6%) and dyspepsia (6%).

Incidence of Taste AEs (All SAD and MAD Cohorts)

	50mg (n=8)	100mg (n=16)	200mg (n=16)	400mg (n=16)	800mg (n=8)	1200 mg (n=8)
Taste Alteration	0 (0%)	1 (6%)	0 (0%)	6 (38%)	5 (63%)	2 (25%)
Partial Taste Loss	0 (0%)	0 (0%)	0 (0%)	1 (6%)	1 (13%)	0 (0%)
Complete Taste Loss	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)

There were no serious adverse events and no healthy volunteers withdrew prematurely due to an adverse event during the trial. No significant trends of mean changes in vital signs, electrocardiogram (ECG) and clinical laboratory values have been observed in the Phase 1 trial of BLU-5937. One subject had a mild elevation of liver enzymes at 400 mg BID that normalized at follow up visit. This increase in liver enzyme levels was not associated with any signs of liver toxicity (e.g., no increase in bilirubin and no clinical symptoms of liver toxicity). There was also a slight increase in bilirubin in some subjects dosed at 400 mg BID. This elevation in bilirubin was not associated with any concomitant increases in liver enzyme levels and returned to baseline value two days after drug discontinuation, which suggests that it is most likely benign and due to an interaction between BLU-5937 and bilirubin hepatic disposition.

Trial Design

The clinical Phase 1 trial was a randomized, double-blind, placebo-controlled trial of orally administered BLU-5937 in 90 healthy adult subjects. The primary objectives of this trial were to assess the safety, tolerability (including taste perception) and pharmacokinetic profile of BLU-5937 in healthy subjects. The trial was divided in two parts:

- **Part 1**. A single ascending dose (SAD) trial was conducted in 60 healthy subjects. Subjects were randomized into six cohorts of 10 subjects (8 BLU-5937: 2 placebo). The trial evaluated single oral doses of BLU-5937 from 50 to 1200 mg.

- **Part 2**. A multiple ascending dose (MAD) trial was conducted in 30 healthy subjects. Subjects were randomized into three cohorts of 10 subjects (8 BLU-5937: 2 placebo). The trial evaluated multiple oral doses of BLU-5937 of 100, 200 and 400 mg administered twice-a-day (BID) for seven consecutive days.

BLU-5937 Regulatory Pathway in Chronic Cough

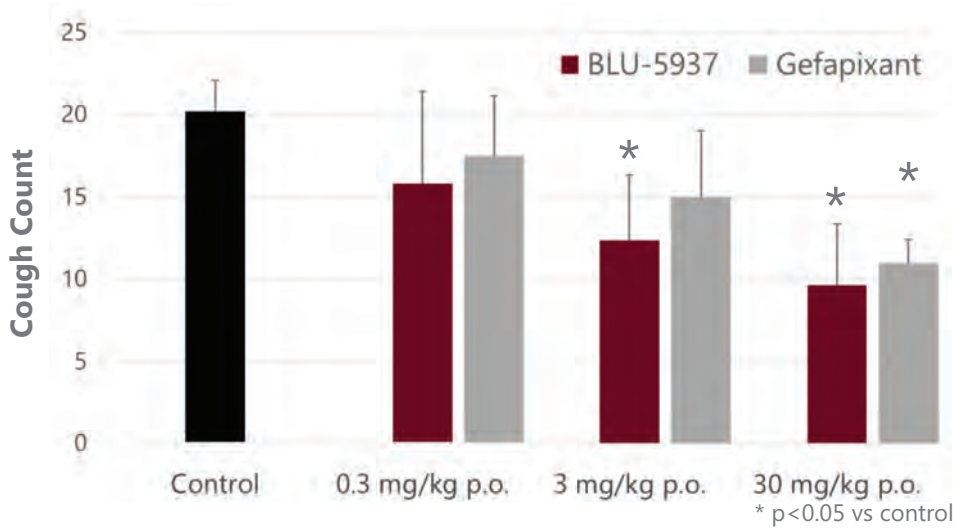
If the results of the RELIEF trial are positive, we expect to meet with the FDA and European regulatory authorities to discuss the registration pathway for BLU-5937 in chronic cough patients and the design of the next trial, including the target population, dose, duration and primary efficacy endpoint. We expect to initiate either a Phase 2b or a Phase 2/3 trial to further pursue the development of BLU-5937 for the treatment of chronic cough. We will then need to conduct an additional Phase 3 clinical trial to support the submission of a new drug application ("**NDA**") to the FDA and a marketing authorization application ("**MAA**") to the European Medicines Agency ("**EMA**") for BLU-5937 in chronic cough. If the results of these studies are positive, we would plan to seek approval for BLU-5937 for refractory chronic cough which, if successful, would lead to the marketing and sale of BLU-5937. See "*Risk Factors*".

BLU-5937 Preclinical Studies

BLU-5937's Reduction in Cough Frequency Comparable to the Leading P2X3 Inhibitor, Gefapixant

The antitussive effect of BLU-5937 was compared to that of gefapixant in a guinea pig cough model. Treatments (control, BLU-5937 (0.3, 3 and 30 mg/kg) or gefapixant (0.3, 3 or 30 mg/kg)) were administered orally in seven groups of six animals two hours prior to tussive agent exposure (citric acid and histamine) and the number of coughs were counted for a period of 15 minutes. Both treatments showed comparable dose-dependent reduction in cough frequency as compared to the control. The reduction in cough was statistically significant at 3 mg/kg (39% vs. control) and 30 mg/kg (52% vs. control) with BLU-5937, and at 30 mg/kg (45% vs. control) with gefapixant.

Guinea Pig Cough Inhibition Study



Treatments (control, BLU-5937, gefapixant) were administered orally (p.o.) 2 hours prior to tussive agent exposure: citric acid (0.1 M, aerosol) and histamine (0.6 mM, aerosol); n=6 animals per group

BLU-5937's Duration of Effect also Comparable to Gefapixant

Using the same guinea pig cough model, a time course study was conducted to assess the duration of the antitussive effect of BLU-5937 and gefapixant following the administration of a single oral 30 mg/kg dose. In this study, animals in groups of six were exposed to tussive agents (citric acid and histamine) at various times after the administration of the study drugs (two, four, six, eight and twelve hours post-dose for BLU-5937 and two and eight hours post-dose for gefapixant) and the number of coughs were measured

for 15 minutes. The reduction in cough frequency compared to control was observed to be statistically significant at two, four and six hours post-dose with BLU-5937, and at two hours post-dose with gefapixant. The antitussive effect was no longer significant at eight hours post-dose for both agents.

BLU-5937 Was not Associated with Taste Loss, Whereas Gefapixant Showed Significant Taste Loss in a Rat Taste Model

A rat taste model was used to compare BLU-5937's effect on taste perception with that of gefapixant. Animals were water-fasted overnight and presented with one bottle of water and one bottle of (bitter-tasting) quinine at the time corresponding to the maximum plasma concentration of study drugs. The volume of liquid consumed from each bottle was measured for 15 minutes. Treatments (control, BLU-5937 (10 or 20 mg/kg) or gefapixant (10 or 20 mg/kg)) were administered intraperitoneally in two groups of 10 rats. Animals treated with BLU-5937 did not drink more quinine than the control animals, while those treated with gefapixant drank significantly (approximately four to five times) more quinine than the control at the two doses tested. These results indicate that BLU-5937 was not associated with taste loss whereas gefapixant led to significant taste loss.

Chronic Pruritus

A Burdensome Condition Effecting Quality of Life

Chronic pruritus, defined as itching lasting longer than six weeks, can be as burdensome as chronic pain in negatively impacting a patient's quality of life. The urge to scratch can be unbearable, and the act of scratching can remove layers of skin and break the skin barrier leading to bleeding, scarring and greatly increasing the risk of infection. Similar to chronic pain, severe chronic pruritus causes a number of physical and psychological issues that substantially impact patients' day-to-day wellbeing. Chronic pruritus can lead to trouble sleeping, resulting in loss of work productivity and increased anxiety and depression.

Chronic pruritus is a hallmark of many conditions, including atopic dermatitis. It is estimated that there are 16.9 million adults in the United States who have atopic dermatitis, a chronic, inflammatory skin disease that is most commonly first diagnosed in childhood. Atopic dermatitis is characterized by skin barrier disruption and immune dysregulation. Patients with atopic dermatitis may have chronically inflamed skin lesions and often have persistent pruritus. Physicians and patients report pruritus as the primary patient complaint associated with this disease. Of the total population of adults affected by atopic dermatitis in the United States, it is estimated that three million of those are actually diagnosed with the disease, and of those diagnosed, it is estimated that 2.25 million of these patients are actively being treated by a physician.

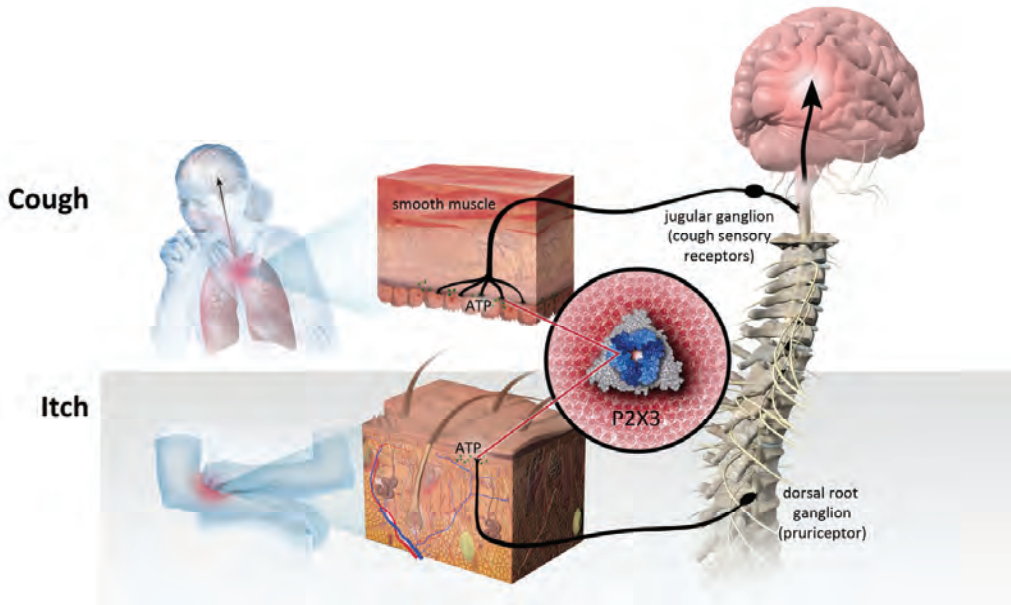
For people suffering with atopic dermatitis, the quality of life impact of the disease is multifaceted and can be constant. Much of this impact is related to its major symptom, itch, its effect on sleep, its outward visibility and the expense and time-consuming nature of prescription and topical treatments. Atopic dermatitis affects social, sexual, academic and occupational functioning and is also associated with increased rates of depression and anxiety.

Creams and ointments and topical corticosteroids or other topical or systemic anti-inflammatory agents are routinely used to manage skin health and to reduce skin inflammation in patients with atopic dermatitis. However, despite currently available treatments, an estimated 40-50% of atopic dermatitis patients report having inadequate relief of their pruritus and are in need of new, efficacious pruritus therapies.

BLU-5937: A Promising Potential Therapy for Chronic Pruritus

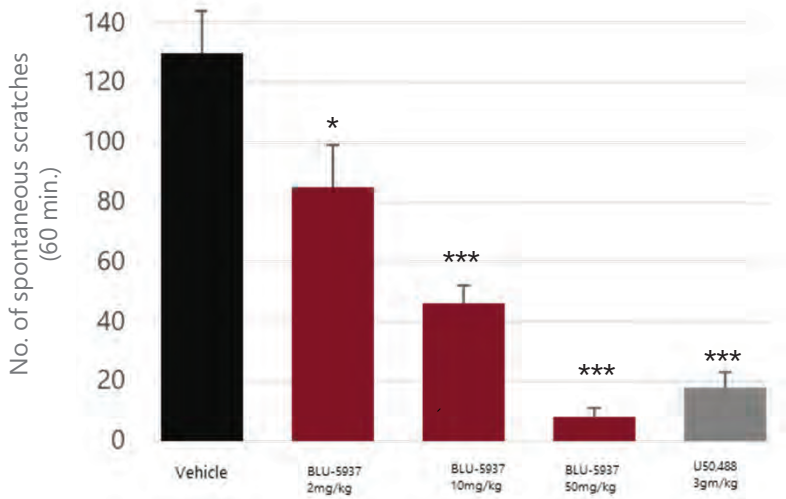
Based on similarities between the manifestation of the symptoms between cough and itch, we believe that BLU-5937 may be a promising, novel therapeutic modality for chronic pruritus associated with atopic dermatitis. Neuronal terminals in the skin are known to express P2X3 receptors and the hypersensitization of afferent neurons expressing P2X3 receptors may also be involved in chronic pruritus. We believe that increased release of ATP in atopic dermatitis leads to hyperexcitability of afferent pruriceptive neurons mediated by P2X3 receptors leading to pruritus. We believe BLU-5937, a potent and selective P2X3 inhibitor, therefore has the potential to address chronic pruritus associated with atopic dermatitis.

Mechanistic Similarities Between Cough and Itch



Preclinical studies conducted by us provided evidence that the ATP-induced hypersensitization mediated by P2X3 receptors in cutaneous C-fibers plays a key role in pruritus. In multiple animal models of pruritus, we observed that treatment with BLU-5937 resulted in significant anti-pruritic effect. As shown in the figure below, BLU-5937 was evaluated in the calcipotriol-induced murine model of atopic dermatitis where it was observed to result in potent, statistically-significant and dose-dependent reductions of spontaneous scratching compared to placebo. These studies formed the basis for our clinical development plan in chronic pruritus.

Atopic Dermatitis Mouse Model



Number of spontaneous scratches in 60 min of day 8 Calcipotriol (MC903) treated mice pre-injected with vehicle, 2, 10, or 50 mg/kg test BLU-5937, or 3 mg/kg U50,488. (n = 10 mice per group),*p < 0,05, ***p < 0.0001, one-way ANOVA. Data are represented as mean ± S.E.M. U50,488: kappa opioid agonist

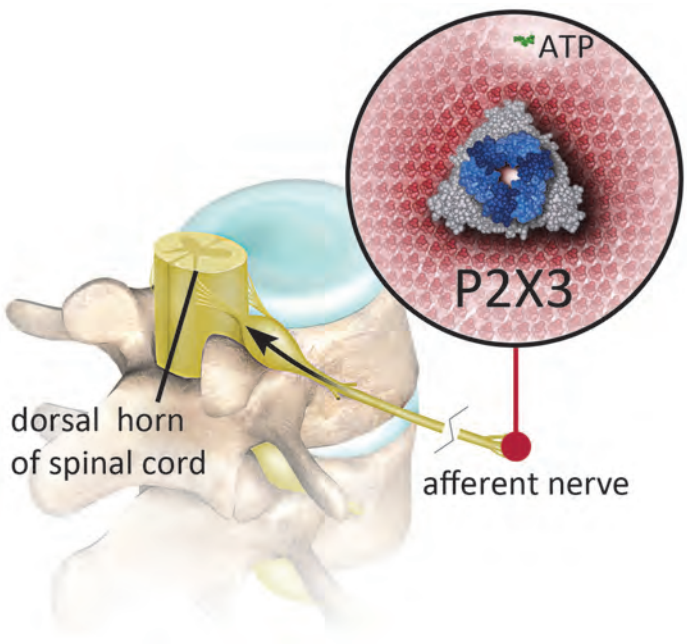
Clinical Development Plan

We plan to initiate a Phase 2 clinical trial of BLU-5937 in chronic pruritus in Q2 2020, with topline data expected in mid-2021. This Phase 2 clinical trial is expected to be a randomized, double-blind, placebo-controlled and parallel group design trial enrolling approximately 100 patients suffering from moderate to severe chronic pruritus associated with mild to moderate atopic dermatitis. The trial is expected to be a two-arm study comparing BLU-5937 to placebo, each administered orally, BID for four weeks. The primary efficacy endpoint will be the change from baseline to week four in the Worst Itch Numeric Rating Scale, or "WI-NRS".

BLU-5937 in Other P2X3 Hypersensitization-Related Disorders

We believe BLU-5937, a potent and highly selective P2X3 inhibitor, has the potential to be an important treatment option for chronic cough, chronic pruritus and other P2X3 hypersensitization-related disorders. To further elucidate the therapeutic potential of BLU-5937 beyond chronic cough, we have initiated a review of pathologies involving aberrant ATP-P2X3 signaling resulting in hypersensitization. Further to our review and preclinical in vitro and in vivo studies, we are pursuing BLU-5937 as a treatment of chronic pruritus, or chronic itch, associated with atopic dermatitis. In addition to chronic couch and chronic pruritus, BLU-5937 may also have broad applicability across other afferent hypersensitization-related disorders, potentially enabling us to build a pipeline of therapies using our P2X3 platform. We are evaluating the potential role of P2X3 inhibition in the treatment of these other afferent hypersensitization-related disorders, including those associated with irritation and pain.

P2X3 Sensitization Contributes to Irritation and Pain



Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary protection for BLU-5937 and its therapeutic applications, in order to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing U.S. and foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trade secrets, know-how and continuing technological innovation to further develop and maintain our proprietary position.

Composition of matter patent coverage for BLU-5937 has been secured in all major pharmaceutical markets: the United States of America, Europe, Japan and China. Patents issued have claims covering the

composition of matter of BLU-5937 and related imidazopyridine compounds and uses thereof. The patents have an expiration date of 2034, excluding any potential patent term extension. Patent applications with similarly broad claims are currently pending in other industrialized nations.

In addition, the USPTO has issued patent No. 10,111,883 granting claims for the use of BLU-5937 for the treatment of chronic cough without affecting taste response. More generally, this patent claims the use of imidazopyridine compounds, including BLU-5937, that are selective for the P2X3 receptor as a means of minimizing taste perturbation in patients treated for chronic cough. Patent No. 10,111,883 has an expiration date of 2038, excluding any potential patent term extension. This new U.S. patent extends the patent protection of BLU-5937 by an additional four years, to 2038.

In addition to patent protection granting claims to composition of matter, our patent estate also includes patents and patent applications associated with the use of BLU-5937 and related compounds as a treatment for various hypersensitization disorders, including chronic cough and chronic pruritus.

The terms of individual patents depend upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, the patent term is generally 20 years from the earliest claimed filing date of a nonprovisional patent application in the applicable country. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over a commonly owned patent or a patent naming a common inventor and having an earlier expiration date. The Drug Price Competition and Patent Term Restoration Act of 1984, or the "Hatch-Waxman Act", permits a patent term extension of up to five years beyond the expiration date of a U.S. patent as partial compensation for the length of time the drug is under regulatory review while the patent is in force. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. We cannot provide any assurance that any patent term extension with respect to any U.S. patent will be obtained and, if obtained, the duration of such extension.

Similar provisions are available in the European Union and certain other non-U.S. jurisdictions to extend the term of a patent that covers an approved drug. In the future, if BLU-5937 receives approval from the FDA or non-U.S. regulatory authorities, we expect to apply for patent term extensions on issued patents covering BLU-5937, depending upon the length of the clinical trials for BLU-5937 and other factors. The expiration dates referred to above are without regard to potential patent term extension or other market exclusivity that may be available to us. However, we cannot provide any assurances that any such patent term extension of a non-U.S. patent will be obtained and, if obtained, the duration of such extension.

We also protect our proprietary technology and processes, in part, by confidentiality and invention assignment agreements with our employees, consultants, scientific advisors and other contractors. These agreements may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our employees, consultants, scientific advisors or other contractors use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our commercial success will also depend in part on not infringing the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, alter our processes, obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize BLU-5937 or any future product candidate may have a material adverse impact on us. If third parties prepare and file patent applications that also claim technology to which we have rights, we may have to participate in interference or derivation proceedings to determine priority of invention. See "*Risk Factors*".

REGULATORY MATTERS

Government Regulation and Product Approvals

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as BLU-5937. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidate.

U.S. Government Regulation of Drug Products

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or "FDCA", and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of non-clinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or "GLP", regulations;
- submission to the FDA of an investigational new drug application, or "IND", which must become effective before human clinical trials may begin in the United States;
- approval by an independent institutional review board, or "IRB", at each clinical site before each trial may be initiated;
- performance of adequate and well controlled human clinical trials in accordance with good clinical practice, or "GCP", requirements to establish the safety and efficacy of the proposed drug product for each indication;
- submission to the FDA of an NDA;
- satisfactory completion of an FDA advisory committee meeting, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites and the sponsor's clinical trial records to assure compliance with GCP requirements and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or "REMS", and the potential requirement to conduct post approval studies.

Non-clinical Studies

Non-clinical studies include laboratory evaluation of product chemistry and formulation, as well as animal studies to assess safety, toxicity and efficacy. The conduct of the non-clinical tests must comply with federal regulations and requirements, including GLPs. An IND sponsor must submit the results of the

non-clinical tests, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. Some non-clinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human patients under the supervision of qualified investigators in accordance with GCP requirements, which include the requirement that all research patients provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each institution participating in the clinical trial must review and approve the protocol for any clinical trial including informed consent information before the trial commences at that institution. Information about most clinical trials must be submitted within specific timeframes for publication on the www.clinicaltrials.gov website. Information related to the product, patient population, phase of investigation, trial sites and investigators, and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed in some cases for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human patients or patients with the target disease or condition and tested for safety, dosage tolerance, pharmacokinetics, absorption, metabolism, distribution, excretion, side effects and, if possible, to gain an early indication of its effectiveness.
- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. In most cases, FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate efficacy of the drug.
- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk benefit profile of the product, and to provide adequate information for the labeling of the product.
- Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. The FDA or the sponsor may suspend or terminate a clinical trial at any time or the FDA may impose other sanctions on various grounds, including a finding that the research patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the non-clinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is

subject to a substantial application user fee. Under the Prescription Drug User Fee Act, or "PDUFA", guidelines that are currently in effect, the FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that the NDA is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to certain performance goals in the review of NDAs. Most applications for standard review drug products are reviewed within ten to twelve months; most NDAs for priority review drugs are reviewed in six to eight months. The review process for both standard and priority review may be extended by FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

In accordance with the Pediatric Research and Equity Act, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements.

The FDA also may require submission of a REMS plan if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks. The REMS plan could include medication guides, physician communication plans, assessment plans and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools.

The FDA may refer an application for a novel drug, or a drug that presents difficult questions of safety or efficacy, to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites and the sponsor to assure compliance with GCP requirements and the integrity of the clinical data submitted in an NDA.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA will issue an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or non-clinical testing in a resubmission to the NDA in order for the FDA to reconsider the application. FDA has committed to reviewing such submissions in two or six months depending on the type of information included in the resubmission. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms

under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, substantial annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data. These fees are typically increased annually.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval or clearance of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or clinical holds on post approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

The Hatch-Waxman Act

Section 505 of the FDCA describes three types of applications that may be submitted to request marketing authorization for a new drug. A 505(b)(1) NDA is an application that contains full reports of

investigations of safety and effectiveness. The Hatch-Waxman Act created two additional marketing pathways under Sections 505(j) and 505(b)(2) of the FDCA. Section 505(j) establishes an abbreviated approval process for generic versions of approved drug products through the submission of an abbreviated new drug application, or "ANDA". An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the branded reference drug and has been observed to be bioequivalent to the branded reference drug. ANDA applicants are required to conduct bioequivalence testing to confirm chemical and therapeutic equivalence to the branded reference drug. Generic versions of drugs can often be substituted by pharmacists under prescriptions written for the branded reference drug.

A 505(b)(2) NDA is an application that contains full reports of investigations of safety and effectiveness but where at least some of the information required for approval comes from studies not conducted by or for the applicant. This alternate regulatory pathway enables the applicant to rely, in part, on the FDA's findings of safety and efficacy for an existing product, or published literature, in support of its application. The FDA may then approve the new product candidate for all or some of the labeled indications for which the branded reference drug has been approved, as well as for any new indication sought by the 505(b)(2) applicant.

The Hatch-Waxman Act establishes periods of regulatory exclusivity for certain approved drug products. The holder of an NDA may obtain five years of exclusivity upon approval of a new drug containing a new chemical entity, or "NCE", that has not been previously approved by the FDA. During the five year exclusivity period, the FDA cannot accept for filing or approve any ANDA seeking approval of a generic version of that drug or any 505(b)(2) NDA for the same active moiety and that relies on the FDA's findings regarding that drug, except that FDA may accept an application for filing (but still may not approve it) after four years if the follow-on applicant makes a paragraph IV certification, as described below. The Hatch-Waxman Act also provides three years of marketing exclusivity to the holder of an NDA for a particular condition of approval, or change to a marketed product, such as a new formulation or new indication for a previously approved product, if one or more new clinical studies (other than bioavailability or bioequivalence studies) was essential to the approval of the application and was conducted/sponsored by the applicant. This three-year exclusivity period protects against FDA approval of ANDAs and 505(b)(2) NDA for drugs that include the innovation that required the new clinical data.

Upon submission of an ANDA or a 505(b)(2) NDA, an applicant must certify to the FDA, in the opinion of the applicant and to the best of its knowledge (1) that relevant patent information on the referenced drug product has not been submitted to the FDA; (2) that the relevant patent has expired; (3) the date on which the relevant patent expires; or (4) that such patent is invalid, unenforceable or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. If the NDA holder or patent owner(s) files a patent infringement action against the ANDA or 505(b)(2) applicant within 45 days of receipt of the paragraph IV certification, the FDA may not approve the ANDA or 505(b)(2) application until the earlier of (i) 30 months from the receipt of the notice of the paragraph IV certification (generally referred to as the 30 month stay), (ii) the expiration date of the patent(s) listed in the Orange Book for the reference drug product, (iii) the date the court enters a final order or judgment that the patent(s) are invalid, unenforceable and/or not infringed or (iv) such shorter or longer period as may be ordered by a court. Where the ANDA or 505(b)(2) applicant files an application with a paragraph IV certification within the fifth year of the five-year NCE exclusivity period enjoyed by the NDA holder for the reference branded product, and where patent litigation is brought within 45 days of receipt of notice of the paragraph IV certification, the 30-month stay will be extended by the amount of time such that 7.5 years will elapse from the date of approval of the original NDA to the expiration of the stay. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes, whether the reference product enjoys NCE exclusivity, and the reference drug sponsor's decision to initiate patent litigation. However, an ANDA applicant may be able to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

Regulation Outside the United States

In the European Economic Area, or "EEA", which is composed of the 28 Member States of the European Union plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or "MA".

There are two types of MAs:

- The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use, or "CHMP", of the European Medicines Agency, or "EMA", and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products and medicinal products that contain a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU. Under the Centralized Procedure the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when the authorization of a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. Under the accelerated procedure the standard 210 days review period is reduced to 150 days.

- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member State through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure.

Prior to obtaining an MA in the EEA, applicants have to demonstrate compliance with all measures included in a Paediatric Investigation Plan, or "PIP", approved by the EEA regulatory agency, covering all subsets of the pediatric population, unless the EEA regulatory agency has granted (1) a product-specific waiver, (2) a class waiver or (3) a deferral for one or more of the measures included in the PIP.

In the EEA, upon receiving an MA, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the EEA from referencing the innovator's data to assess a generic application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the EEA regulatory agencies to be a new chemical entity, and products may not qualify for data exclusivity.

Other Healthcare Laws

In addition to FDA restrictions on the marketing of pharmaceutical products, other foreign, federal and state healthcare regulatory laws restrict business practices in the pharmaceutical industry. These laws include, but are not limited to, federal and state anti-kickback, false claims, data privacy and security, and physician payment and drug pricing transparency laws.

The U.S. federal Anti-Kickback Statute prohibits, among other things, any person or entity from knowingly and willfully offering, paying, soliciting, receiving or providing any remuneration, directly or indirectly, overtly or covertly, to induce or in return for purchasing, leasing, ordering, or arranging for or recommending the purchase, lease, or order of any good, facility, item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federal healthcare programs. The term "remuneration" has been broadly interpreted to include anything of value. The Anti-Kickback Statute has been interpreted to apply

to arrangements between pharmaceutical and medical device manufacturers on the one hand and prescribers, purchasers, formulary managers and beneficiaries on the other hand. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not meet the requirements of a statutory or regulatory exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the U.S. federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the statute has been violated. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Moreover, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. The majority of states also have anti-kickback laws, which establish similar prohibitions and in some cases may apply to items or services reimbursed by any third-party payor, including commercial insurers.

The federal false claims and civil monetary penalties laws, including the civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false, fictitious or fraudulent claim for payment to, or approval by, the federal government, knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. Actions under the civil False Claims Act may be brought by the Attorney General or as a *qui tam* action by a private individual in the name of the government. Violations of the civil False Claims Act can result in very significant monetary penalties and treble damages. Several pharmaceutical, medical device and other healthcare companies have been prosecuted under these laws for, among other things, allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of products for unapproved (e.g., or off-label) uses. In addition, the civil monetary penalties statute imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many states also have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Violations of fraud and abuse laws, including federal and state anti-kickback and false claims laws, may be punishable by criminal and civil sanctions, including significant fines and civil monetary penalties, the possibility of exclusion from federal healthcare programs (including Medicare and Medicaid), disgorgement and the implementation of corporate integrity agreements, which impose, among other things, rigorous operational and monitoring requirements on companies. Similar sanctions and penalties, as well as imprisonment and exclusion from federal healthcare programs, also can be imposed upon executive officers and employees of such companies. It is expected that the government authorities will continue to devote substantial resources to investigating healthcare providers' and manufacturers' compliance with applicable fraud and abuse laws.

The federal Health Insurance Portability and Accountability Act of 1996, or "HIPAA", created additional federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

In addition, there has been a recent trend of increased federal and state regulation of payments made to physicians and certain other healthcare providers. The Affordable Care Act imposed, among other things, new annual reporting requirements through the Physician Payments Sunshine Act for covered manufacturers for certain payments and "transfers of value" provided to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Failure to submit timely, accurately and completely the required information for all payments, transfers of value and ownership or investment interests may result in civil monetary penalties. Covered manufacturers must submit reports by the 90th day of each subsequent calendar year and the reported information is publicly made available on a searchable website. In addition, certain states require implementation of compliance programs and compliance with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, impose restrictions on marketing practices and/or require the tracking and reporting of marketing expenditures and pricing information as well as gifts, compensation and other remuneration or items of value provided to physicians and other healthcare professionals and entities.

We may also be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or "HITECH", and their respective implementing regulations, including the Final HIPAA Omnibus Rule published on January 25, 2013, impose specified requirements relating to the privacy, security and transmission of individually identifiable health information held by covered entities and their business associates. Among other things, HITECH made HIPAA's security standards directly applicable to "business associates," defined as independent contractors or agents of covered entities that create, receive, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same requirements, thus complicating compliance efforts.

If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to significant civil, criminal, and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws.

Similar state and foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or other transfers of value to healthcare professionals, may apply to us to the extent that any of our product candidates, once approved, are sold in a country other than the United States.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical product for which we obtain regulatory approval. In the United States and markets in other countries, patients who are prescribed drugs generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Providers and patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. If approved, sales of BLU-5937 will depend, in part, on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities, managed care plans, private health insurers and other organizations.

In the United States, the process for determining whether a third-party payor will provide coverage for a pharmaceutical product typically is separate from the process for setting the price of such product or for establishing the reimbursement rate that the payor will pay for the product once coverage is approved. With

respect to drugs, third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication, or place products at certain formulary levels that result in lower reimbursement levels and higher cost-sharing obligation imposed on patients. A decision by a third-party payor not to cover a product could reduce physician utilization of a product. Moreover, a third-party payor's decision to provide coverage for a pharmaceutical product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable a manufacturer to maintain price levels sufficient to realize an appropriate return on its investment in product development. Additionally, coverage and reimbursement for products can differ significantly from payor to payor. One third-party payor's decision to cover a particular medical product does not ensure that other payors will also provide coverage for the medical product, or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process usually requires manufacturers to provide scientific and clinical support for the use of their products to each payor separately and is a time-consuming process.

In the European Union, governments influence the price of products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription products, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross border imports from low-priced markets exert a commercial pressure on pricing within a country.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of pharmaceutical products have been a focus in this effort. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of pharmaceutical products, in addition to questioning safety and efficacy. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover that product after FDA approval or, if they do, the level of payment may not be sufficient to allow a manufacturer to sell its product at a profit.

Healthcare Reform and Other Potential Changes to Healthcare Laws

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, in December 2016, the 21st Century Cures Act, or "Cures Act", was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and devices and to spur innovation, but its ultimate implementation is unclear. In addition, in August 2017, the FDA Reauthorization Act was signed into law, which reauthorized the FDA's user fee programs and included additional drug and device provisions that build on the Cures Act. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products and services, implementing reductions in Medicare and other healthcare funding, and applying new payment methodologies. For example, in March 2010, the Affordable Care Act was enacted, which, among other things, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care plans;

imposed mandatory discounts for certain Medicare Part D beneficiaries as a condition for manufacturers' outpatient drugs coverage under Medicare Part D; subjected drug manufacturers to new annual fees based on pharmaceutical companies' share of sales to federal healthcare programs; imposed a new federal excise tax on the sale of certain medical devices; created a new Patient Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare Innovation at the Center for Medicare & Medicaid Services, or "CMS" to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

Since its enactment, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act, and we expect there will be additional challenges and amendments to the Affordable Care Act in the future. On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Cuts and Jobs Act of 2017. While the Texas U.S. District Court Judge, as well as the current administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business.

In addition, we cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, in the United States, certain policies of the current administration may impact our business and industry. Namely, the current administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act to reduce healthcare expenditures. These changes include the Budget Control Act of 2011, which led to aggregate reductions of Medicare payments to providers of 2% per fiscal year and that will remain in effect through 2027 unless additional action is taken by Congress; the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years; and the Medicare Access and CHIP Reauthorization Act of 2015, which ended the use of the statutory formula for Medicare payment adjustments to physicians, and provided for a 0.25% annual increase in payment rates under the Medicare Physician Fee Schedule through 2019, but no annual update from 2020 through 2025. More recently, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products. In addition, the current administration's budget proposals for fiscal years 2019 and 2020 contain further drug price control measures that could be enacted during the budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Further, the current administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers.

Individual states in the United States have also become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

CONSOLIDATED CAPITALIZATION

Except as otherwise disclosed in this prospectus, there have been no material changes in our consolidated share and loan capital, on a consolidated basis, from September 30, 2019 to the date of this prospectus, other than for 512,222 stock options granted under the stock option plan on November 13, 2019.

Our authorized capital consists of an unlimited number of common shares and an unlimited number of preferred shares, issuable in series. As at January 16, 2020, we had 55,378,660 common shares issued and outstanding, all of which are fully paid and non-assessable, and 60,277,193 common shares on a fully diluted basis, including 4,726,943 stock options granted under the stock option plan and 171,590 broker warrants.

USE OF PROCEEDS

The use of proceeds for any particular offering of common shares under this prospectus will be described in the applicable prospectus supplement. Unless otherwise specified therein, we intend to use the net proceeds of any offering under this prospectus to fund research and development activities, working capital, acquisitions, debt repayment or other general corporate purposes. The aggregate proceeds from the issuance and sale of securities under this prospectus shall not exceed US$250,000,000. We will not receive any proceeds from any sale of our common shares by selling shareholders under this prospectus.

Negative Cash Flow

The Company has incurred significant operating losses and negative cash flows from operations since its inception and has an accumulated deficit of Cdn$509.4 million as at September 30, 2019. Our ability to continue as a going concern is dependent upon raising additional financing through equity and non-dilutive funding and partnerships. There can be no assurance that we will have sufficient capital to fund our ongoing operations or to develop or commercialize any products without future financings. If we are unable to obtain additional financing when required, we may need to substantially reduce or eliminate planned expenditures or be unable to continue operations. Our ability to continue as a going concern is dependent upon our ability to fund research and development programs and defend our patent rights. We anticipate that we will continue to have negative cash flow for the foreseeable future and expect that any proceeds from the sale of securities under this prospectus will be used to fund anticipated negative cash flow from operating activities, as described above.

September 2019 Offering

In September 2019, concurrently with the listing of our common shares on NASDAQ, we completed an equity offering of 11,179,451 of our common shares in Canada and the United States (the "**September 2019 Offering**"), for total gross proceeds of approximately Cdn$104.6 million (US$79.4 million).

We intend to use the net proceeds from the September 2019 Offering, together with our cash, cash equivalents and short-term investments on hand, primarily to fund research and development activities, general and administrative expenses, working capital needs and other general corporate purposes, as indicated in our prospectus supplement dated September 4, 2019.

SELLING SHAREHOLDERS

Common shares may be sold under this prospectus by way of secondary offering by or for the account of certain of our shareholders. The prospectus supplement that will be filed in connection with any offering of our common shares by one or more selling shareholders will include the following information:

- the name or names of the selling shareholders;
- the number or amount of common shares owned, controlled or directed by each selling shareholder;
- the number or amount of common shares being distributed for the account of each selling shareholder;

- the number or amount of common shares of any class to be owned, controlled or directed by the selling shareholder after the distribution and the percentage that number or amount represents of the total number of our outstanding common shares;
- whether the common shares are owned by the selling shareholders both of record and beneficially, of record only, or beneficially only; and
- all other information that is required to be included in the applicable prospectus supplement.

PLAN OF DISTRIBUTION

We may from time to time during the 25-month period that this prospectus, including any amendments hereto, remains valid, offer for sale and issue up to an aggregate of US$250,000,000 common shares. The Company may offer and sell the common shares to or through underwriters, agents, or dealers purchasing as principals, and may also sell directly to one or more purchasers or through agents or pursuant to applicable statutory exemptions.

This prospectus may also, from time to time, relate to the offering of our common shares by certain selling shareholders. The selling shareholders may sell all or a portion of our common shares beneficially owned by them and offered thereby from time to time directly or through one or more underwriters, broker-dealers or agents. Our common shares may be sold by the selling shareholders in one or more transactions at fixed prices (which may be changed from time to time), at market prices prevailing at the time of the sale, at varying prices determined at the time of sale, at prices related to prevailing market prices or at negotiated prices.

The prospectus supplement relating to any particular offering of common shares under this prospectus will identify each underwriter, dealer or agent, as the case may be, engaged by us in connection with such offering and the name or names of any selling shareholders. The prospectus supplement will also set forth the terms of the offering, including, where applicable, any fees, commissions, discounts or any other compensation payable by us or the selling shareholders to underwriters, dealers or agents in connection with the offering, the method of distribution of securities, the initial issue price, the proceeds to us or any selling shareholder and any other material terms of the plan of distribution. Any initial offering price and discounts, concessions or commissions allowed or re-allowed or paid to dealers may be changed from time to time.

The securities may be sold from time to time in one or more transactions at a fixed price or prices or at prices which may be changed or at market prices prevailing at the time of sale, at prices related to such prevailing prices or at negotiated prices, including sales in transactions that are deemed to be "at-the-market distributions" as defined in National Instrument 44-102 — *Shelf Distributions*, including sales made directly on the TSX, NASDAQ or other existing trading markets for the common shares. Any such transactions that are deemed "at-the-market-distributions" will be subject to regulatory approval. No underwriter, dealer or agent, no affiliate of such an underwriter, dealer or agent and no person acting jointly or in concert with such an underwriter, dealer or agent involved in an "at-the-market distribution" will over-allot common shares in connection with such distribution or effect any other transactions that are intended to stabilize or maintain the market price of the securities.

The price at which our common shares will be offered and sold may vary from purchaser to purchaser and during the period of distribution.

In connection with the sale of the securities, underwriters, dealers or agents may receive compensation, including in the form of underwriters', dealers' or agents' fees, commissions or concessions. Underwriters, dealers and agents that participate in the distribution of the securities may be deemed to be underwriters for the purposes of applicable Canadian securities legislation and any compensation received by them from the Company and any profit on the resale of the securities by them may be deemed to be underwriting commissions. In connection with any offering of common shares, except as otherwise set out in a prospectus supplement relating to a particular offering of common shares hereunder and other than in relation to an "at-the-market" distribution, the underwriters, dealers or agents, as the case may be, may over-allot or effect transactions intended to fix, stabilize, maintain or otherwise affect the market price of the common shares at a level other than those which otherwise might prevail on the open market. Such transactions may be commenced, interrupted or discontinued at any time.

Underwriters, dealers or agents who participate in the distribution of the common shares may be entitled, under agreements to be entered into with us, to indemnification the Company against certain liabilities, including liabilities under Canadian securities legislation and the U.S. Securities Act, or to contribution with respect to payments which such underwriters, dealers or agents may be required to make in respect thereof. Such underwriters, dealers and agents may be customers of, engage in transactions with, or perform services for, the Company in the ordinary course of business.

CERTAIN CANADIAN AND UNITED STATES FEDERAL INCOME TAX CONSIDERATIONS

In addition to those Canadian federal income tax considerations described below under "Material Canadian Federal Income Tax Considerations", a prospectus supplement relating to a particular offering of our common shares may also describe certain Canadian federal income tax consequences for an investor acquiring the common shares offered thereunder, including, for investors who are non-residents of Canada, whether the payments of principal, interest or distributions, if any, on the securities will be subject to Canadian non-resident withholding tax.

Moreover, in addition to those U.S. federal income tax considerations described below under "Material United States Federal Income Tax Considerations for U.S. Holders" , the applicable prospectus supplement may also describe certain U.S. federal income tax consequences of the acquisition, ownership and disposition of any common shares offered thereunder by an initial investor who is a U.S. person (within the meaning of the U.S. Internal Revenue Code of 1986, as amended).

Prospective investors should consult their own tax advisers prior to deciding to purchase any of our common shares.

MATERIAL CANADIAN FEDERAL INCOME TAX CONSIDERATIONS

The following is, as of the date hereof, a summary of the principal Canadian federal income tax considerations generally applicable under the *Income Tax Act* (Canada) and the regulations promulgated thereunder, or the "Tax Act", to a holder who acquires, as beneficial owner, common shares in any offering under this prospectus, and who, for purposes of the Tax Act and at all relevant times: (i) holds the common shares as capital property; (ii) deals at arm's length with, and is not affiliated with, us or the underwriters; (iii) is not, and is not deemed to be resident in Canada; and (iv) does not use or hold and will not be deemed to use or hold, our common shares in a business carried on in Canada, or a Non-Resident Holder. Generally, our common shares will be considered to be capital property to a Non-Resident Holder provided the Non-Resident Holder does not hold our common shares in the course of carrying on a business of trading or dealing in securities and has not acquired them in one or more transactions considered to be an adventure or concern in the nature of trade. Special rules, which are not discussed in this summary, may apply to a Non-Resident Holder that is an insurer that carries on an insurance business in Canada and elsewhere. Such Non-Resident Holders should seek advice from their own tax advisors.

This summary is based upon the provisions of the Tax Act in force as of the date hereof, all specific proposals, or the "Proposed Amendments", to amend the Tax Act that have been publicly and officially announced by or on behalf of the Minister of Finance (Canada) prior to the date hereof and Counsels' understanding of the current administrative policies and practices of the Canada Revenue Agency, or the "CRA", published in writing by it prior to the date hereof. This summary assumes the Proposed Amendments will be enacted in the form proposed. However, no assurance can be given that the Proposed Amendments will be enacted in their current form, or at all. This summary is not exhaustive of all possible Canadian federal income tax considerations and, except for the Proposed Amendments, does not take into account or anticipate any changes in the law or any changes in the CRA's administrative policies or practices, whether by legislative, governmental or judicial action or decision, nor does it take into account or anticipate any other federal or any provincial, territorial or foreign tax considerations, which may differ significantly from those discussed herein.

Non-Resident Holders should consult their own tax advisors with respect to an investment in our common shares. This summary is of a general nature only and is not intended to be, nor should it be construed to be, legal or tax advice to any prospective purchaser or holder of our common shares, and no

representations with respect to the income tax consequences to any prospective purchaser or holder are made. Consequently, prospective purchasers or holders of our common shares should consult their own tax advisors with respect to their particular circumstances.

Currency Conversion

Generally, for purposes of the "Tax Act", all amounts relating to the acquisition, holding or disposition of our common shares must be converted into Canadian dollars based on the exchange rates as determined in accordance with the Tax Act. The amounts subject to withholding tax and any capital gains or capital losses realized by a Non-Resident Holder may be affected by fluctuations in the Canadian-U.S. dollar exchange rate.

Disposition of Common Shares

A Non-Resident Holder will not generally be subject to tax under the Tax Act on a disposition of a common share, unless the common share constitutes "taxable Canadian property" (as defined in the Tax Act) of the Non-Resident Holder at the time of disposition and the Non-Resident Holder is not entitled to relief under an applicable income tax treaty or convention.

Provided the common shares are listed on a "designated stock exchange", as defined in the Tax Act (which currently includes the TSX and NASDAQ) at the time of disposition, the common shares will generally not constitute taxable Canadian property of a Non-Resident Holder at that time, unless at any time during the 60-month period immediately preceding the disposition the following two conditions are satisfied concurrently: (i) (a) the Non-Resident Holder; (b) persons with whom the Non-Resident Holder did not deal at arm's length; (c) partnerships in which the Non-Resident Holder or a person described in (b) holds a membership interest directly or indirectly through one or more partnerships; or (d) any combination of the persons and partnerships described in (a) through (c), owned 25% or more of the issued shares of any class or series of our shares; and (ii) more than 50% of the fair market value of our shares was derived directly or indirectly from one or any combination of: real or immovable property situated in Canada, "Canadian resource properties", "timber resource properties" (each as defined in the Tax Act), and options in respect of, or interests in or for civil law rights in, such properties. Notwithstanding the foregoing, in certain circumstances set out in the Tax Act, the common shares could be deemed to be taxable Canadian property. Even if the common shares are taxable Canadian property to a Non-Resident Holder, such Non-Resident Holder may be exempt from tax under the Tax Act on the disposition of such common shares by virtue of an applicable income tax treaty or convention. A Non-Resident Holder contemplating a disposition of common shares that may constitute taxable Canadian property should consult a tax advisor prior to such disposition.

Receipt of Dividends

Dividends received or deemed to be received by a Non-Resident Holder on our common shares will be subject to Canadian withholding tax under the Tax Act. The general rate of withholding tax is 25%, although such rate may be reduced under the provisions of an applicable income tax convention between Canada and the Non-Resident Holder's country of residence. For example, under the *Canada-United States Income Tax Convention (1980)* as amended, or the Treaty, the rate is generally reduced to 15% where the Non-Resident Holder is a resident of the United States for the purposes of, and is entitled to the benefits of, the Treaty.

MATERIAL UNITED STATES FEDERAL INCOME TAX CONSIDERATIONS FOR U.S. HOLDERS

Subject to the limitations and qualifications stated herein, this discussion sets forth material U.S. federal income tax considerations relating to the acquisition, ownership and disposition by U.S. Holders (as hereinafter defined) of the common shares. The discussion is based on the Code, its legislative history, existing and proposed regulations thereunder, published rulings and court decisions, and the *Canada-United States Income Tax Convention (1980)* as amended, or the Treaty, all as currently in effect and all subject to change at any time, possibly with retroactive effect. This summary applies only to U.S. Holders. This discussion of a U.S. Holder's tax consequences addresses only those persons that acquire common shares pursuant to this prospectus and that hold those common shares as capital assets (generally, property held

for investment). In addition, it does not describe all of the tax consequences that may be relevant in light of a U.S. Holder's particular circumstances, including state and local tax consequences, estate and gift tax consequences, alternative minimum tax consequences, and tax consequences applicable to U.S. Holders subject to special rules, such as:

- banks, insurance companies, and certain other financial institutions;
- U.S. expatriates and certain former citizens or long-term residents of the United States;
- dealers or traders in securities who use a mark-to-market method of tax accounting;
- persons holding common shares as part of a hedging transaction, "straddle," wash sale, conversion transaction or integrated transaction or persons entering into a constructive sale with respect to common shares;
- persons whose "functional currency" for U.S. federal income tax purposes is not the U.S. dollar;
- brokers, dealers or traders in securities, commodities or currencies;
- tax-exempt entities or government organizations;
- S corporations, partnerships, or other entities or arrangements classified as partnerships for U.S. federal income tax purposes;
- regulated investment companies or real estate investment trusts;
- persons who acquired our common shares pursuant to the exercise of any employee stock option or otherwise as compensation;
- persons required to accelerate the recognition of any item of gross income with respect to our common shares as a result of such income being recognized on an applicable financial statement;
- persons holding our common shares in connection with a trade or business, permanent establishment, or fixed base outside the United States; and
- persons who own (directly or through attribution) 10% or more (by vote or value) of our outstanding common shares.

If an entity that is classified as a partnership for U.S. federal income tax purposes holds common shares, the U.S. federal income tax treatment of a partner will generally depend on the status of the partner and the activities of the partnership. Partnerships holding common shares and partners in such partnerships are encouraged to consult their tax advisers as to the particular U.S. federal income tax consequences of holding and disposing of common shares.

A "U.S. Holder" is a holder who, for U.S. federal income tax purposes, is a beneficial owner of common shares and is:

- An individual who is a citizen or resident of the United States;
- a corporation, or other entity taxable as a corporation, created or organized in or under the laws of the United States, any state therein or the District of Columbia;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or (2) the trust has a valid election in effect to be treated as a U.S. person under applicable U.S. Treasury Regulations.

PERSONS CONSIDERING AN INVESTMENT IN COMMON SHARES SHOULD CONSULT THEIR OWN TAX ADVISORS AS TO THE PARTICULAR TAX CONSEQUENCES APPLICABLE TO THEM RELATING TO THE ACQUISITION, OWNERSHIP AND DISPOSITION OF THE COMMON SHARES, INCLUDING THE APPLICABILITY OF U.S. FEDERAL, STATE AND LOCAL TAX LAWS.

Passive Foreign Investment Company Rules

If we are classified as a PFIC in any taxable year, a U.S. Holder will be subject to special rules generally intended to reduce or eliminate any benefits from the deferral of U.S. federal income tax that a U.S. Holder could derive from investing in a non-U.S. company that does not distribute all of its earnings on a current basis.

A non-U.S. corporation will be classified as a PFIC for any taxable year in which, after applying certain look-through rules, either:

- at least 75% of its gross income is passive income (such as interest income); or
- at least 50% of its gross assets (determined on the basis of a quarterly average) is attributable to assets that produce passive income or are held for the production of passive income.

We will be treated as owning our proportionate share of the assets and earning our proportionate share of the income of any other corporation, the equity of which we own, directly or indirectly, 25% or more (by value).

Based on our interpretation of the law, our recent financial statements, and taking into account expectations about our income, assets and activities, we believe that we were a PFIC for the taxable year ended December 31, 2018 and expect that we will be a PFIC for the current taxable year. A separate determination must be made after the close of each taxable year as to whether we are a PFIC for that year, and as a result, our PFIC status may change from year to year. The total value of our assets for purposes of the asset test generally will be calculated using the market price of the common shares, which may fluctuate considerably. Fluctuations in the market price of the common shares may result in our being a PFIC for any taxable year. Because of the uncertainties involved in establishing our PFIC status, there can be no assurance regarding if we currently are treated as a PFIC, or may be treated as a PFIC in the future.

If we are classified as a PFIC in any year with respect to which a U.S. Holder owns the common shares, we will continue to be treated as a PFIC with respect to such U.S. Holder in all succeeding years during which the U.S. Holder owns the common shares, regardless of whether we continue to meet the tests described above unless (i) we cease to be a PFIC and the U.S. Holder has made a "deemed sale" election under the PFIC rules, or (ii) the U.S. Holder makes a Qualified Electing Fund Election, or QEF Election, with respect to all taxable years during such U.S. Holders holding period in which we are a PFIC. If the "deemed sale" election is made, a U.S. Holder will be deemed to have sold the common shares the U.S. Holder holds at their fair market value and any gain from such deemed sale would be subject to the rules described below. After the deemed sale election, so long as we do not become a PFIC in a subsequent taxable year, the U.S. Holder's common shares with respect to which such election was made will not be treated as shares in a PFIC and the U.S. Holder will not be subject to the rules described below with respect to any "excess distribution" the U.S. Holder receives from us or any gain from an actual sale or other disposition of the common shares. U.S. Holders should consult their tax advisors as to the possibility and consequences of making a deemed sale election if we cease to be a PFIC and such election becomes available.

For each taxable year we are treated as a PFIC with respect to U.S. Holders, U.S. Holders will be subject to special tax rules with respect to any "excess distribution" such U.S. Holder receives and any gain such U.S. Holder recognizes from a sale or other disposition (including, under certain circumstances, a pledge) of common shares, unless (i) such U.S. Holder makes a QEF Election or (ii) our common shares constitute "marketable" securities, and such U.S. Holder makes a mark-to-market election as discussed below. Absent the making of a QEF Election or a mark-to-market election, distributions a U.S. Holder receives in a taxable year that are greater than 125% of the average annual distributions a U.S. Holder received during the shorter of the three preceding taxable years or the U.S. Holder's holding period for the common shares will be treated as an excess distribution. Under these special tax rules:

- the excess distribution or gain will be allocated ratably over a U.S. Holder's holding period for the common shares;
- the amount allocated to the current taxable year, and any taxable year prior to the first taxable year in which we became a PFIC, will be treated as ordinary income; and

- the amount allocated to each other year will be subject to the highest tax rate in effect for that year and the interest charge generally applicable to underpayments of tax will be imposed on the resulting tax attributable to each such year.

The tax liability for amounts allocated to years prior to the year of disposition or "excess distribution" cannot be offset by any net operating losses for such years, and gains (but not losses) realized on the sale of the common shares cannot be treated as capital, even if a U.S. Holder holds the common shares as capital assets.

In addition, if we are a PFIC, a U.S. Holder will generally be subject to similar rules with respect to distributions we receive from, and our dispositions of the stock of, any of our direct or indirect subsidiaries that also are PFICs, as if such distributions were indirectly received by, and/or dispositions were indirectly carried out by, such U.S. Holder. U.S. Holders should consult their tax advisors regarding the application of the PFIC rules to our subsidiaries.

If a U.S. Holder makes an effective QEF Election, the U.S. Holder will be required to include in gross income each year, whether or not we make distributions, as capital gains, such U.S. Holder's pro rata share of our net capital gains and, as ordinary income, such U.S. Holder's pro rata share of our earnings in excess of our net capital gains. If we determine that we are a PFIC for this year or any future taxable year, we currently expect that we would provide the information necessary for U.S. Holders to make a QEF Election.

U.S. Holders also can avoid the interest charge on excess distributions or gain relating to the common shares by making a mark-to-market election with respect to the common shares, provided that the common shares are "marketable." Common shares will be marketable if they are "regularly traded" on certain U.S. stock exchanges or on a foreign stock exchange that meets certain conditions. For these purposes, the common shares will be considered regularly traded during any calendar year during which they are traded, other than in de minimis quantities, on at least 15 days during each calendar quarter. Any trades that have as their principal purpose meeting this requirement will be disregarded. Our common shares are listed on NASDAQ, which is a qualified exchange for these purposes. Consequently, if our common shares remain listed on NASDAQ and are regularly traded, and you are a holder of common shares, we expect the mark-to-market election would be available to U.S. Holders if we are a PFIC. Each U.S. Holder should consult its own tax advisor as to the whether a mark-to-market election is available or advisable with respect to the common shares.

A U.S. Holder that makes a mark-to-market election must include in ordinary income for each year an amount equal to the excess, if any, of the fair market value of the common shares at the close of the taxable year over the U.S. Holder's adjusted tax basis in the common shares. An electing holder may also claim an ordinary loss deduction for the excess, if any, of the U.S. Holder's adjusted basis in the common shares over the fair market value of the common shares at the close of the taxable year, but this deduction is allowable only to the extent of any net mark-to-market gains for prior years. Gains from an actual sale or other disposition of the common shares will be treated as ordinary income, and any losses incurred on a sale or other disposition of the shares will be treated as an ordinary loss to the extent of any net mark-to-market gains for prior years. Once made, the election cannot be revoked without the consent of the Internal Revenue Service ("**IRS**"), unless the common shares cease to be marketable.

However, a mark-to-market election generally cannot be made for equity interests in any lower-tier PFICs that we own, unless shares of such lower-tier PFIC are themselves "marketable." As a result, even if a U.S. Holder validly makes a mark-to-market election with respect to our common shares, the U.S. Holder may continue to be subject to the PFIC rules (described above) with respect to its indirect interest in any of our investments that are treated as an equity interest in a PFIC for U.S. federal income tax purposes. U.S. Holders should consult their tax advisors to determine whether any of these elections would be available and if so, what the consequences of the alternative treatments would be in their particular circumstances.

Unless otherwise provided by the United States Treasury Department, or the "U.S. Treasury", each U.S. shareholder of a PFIC is required to file an annual report containing such information as the U.S. Treasury may require. A U.S. Holder's failure to file the annual report will cause the statute of limitations for such U.S. Holder's U.S. federal income tax return to remain open with regard to the items required to be included in such report until three years after the U.S. Holder files the annual report, and, unless such

failure is due to reasonable cause and not willful neglect, the statute of limitations for the U.S. Holder's entire U.S. federal income tax return will remain open during such period. U.S. Holders should consult their tax advisors regarding the requirements of filing such information returns under these rules.

WE STRONGLY URGE YOU TO CONSULT YOUR TAX ADVISOR REGARDING THE IMPACT OF OUR PFIC STATUS ON YOUR INVESTMENT IN THE COMMON SHARES AS WELL AS THE APPLICATION OF THE PFIC RULES TO YOUR INVESTMENT IN THE COMMON SHARES.

Cash Dividends and Other Distributions

Subject to the discussion under "Passive Foreign Investment Company Rules" above, to the extent there are any distributions made with respect to the common shares, a U.S. Holder generally will be required to include in its gross income distributions received with respect to its common shares (including the amount of Canadian taxes withheld, if any) as dividend income, but only to the extent that the distribution is paid out of our current or accumulated earnings and profits (computed using U.S. federal income tax principles), with the excess treated first as a non-taxable return of capital to the extent of the holder's adjusted tax basis in its common shares and, thereafter, as capital gain recognized on a sale or exchange on the day actually or constructively received by the holder (as described below under "Sale or Disposition of Common Shares"). There can be no assurance that we will maintain calculations of our earnings and profits in accordance with U.S. federal income tax accounting principles. U.S. Holders should therefore assume that any distribution with respect to the common shares will constitute ordinary dividend income. Dividends paid on the common shares will not be eligible for the dividends received deduction allowed to U.S. corporations.

Dividends paid to a non-corporate U.S. Holder by a "qualified foreign corporation" may be subject to reduced rates of taxation if certain holding period and other requirements are met. A qualified foreign corporation generally includes a foreign corporation if (i) its common shares are readily tradable on an established securities market in the United States or it is eligible for benefits under a comprehensive U.S. income tax treaty that includes an exchange of information program and which the U.S. Treasury has determined is satisfactory for these purposes and (ii) if such foreign corporation is not a PFIC (as discussed above) for either the taxable year in which the dividend is paid or the preceding taxable year. The common shares are readily tradable on an established securities market, the NASDAQ. We may also be eligible for the benefits of the Treaty. Accordingly, subject to the PFIC rules discussed above, we expect that a non-corporate U.S. Holder should qualify for the reduced rate on dividends so long as the applicable holding period requirements are met. U.S. Holders should consult their own tax advisors regarding the availability of the reduced tax rate on dividends in light of their particular circumstances.

Distributions paid in a currency other than U.S. dollars will be included in a U.S. Holder's gross income in a U.S. dollar amount based on the spot exchange rate in effect on the date of actual or constructive receipt, whether or not the payment is converted into U.S. dollars at that time. The U.S. Holder will have a tax basis in such currency equal to such U.S. dollar amount, and any gain or loss recognized upon a subsequent sale or conversion of the foreign currency for a different U.S. dollar amount will generally be U.S. source ordinary income or loss.

If the dividend is converted into U.S. dollars on the date of receipt, a U.S. Holder generally should generally not be required to recognize foreign currency gain or loss in respect of the dividend income.

If a U.S. Holder is subject to Canadian withholding taxes (at the rate applicable to such U.S. Holder) with respect to dividends paid on the common shares, such U.S. Holder may be entitled to receive either a deduction or a foreign tax credit for such Canadian taxes paid. Complex limitations apply to the foreign tax credit. Dividends paid by us generally will constitute "foreign source" income and generally will be categorized as "passive category income." Because the foreign tax credit rules are complex, each U.S. Holder should consult its own tax advisor regarding the foreign tax credit rules.

Sale or Disposition of Common Shares

A U.S. Holder generally will recognize gain or loss on the taxable sale or exchange of the common shares in an amount equal to the difference between the U.S. dollar amount realized on such sale or exchange (determined in the case of the common shares sold or exchanged for currencies other than U.S.

dollars by reference to the spot exchange rate in effect on the date of the sale or exchange or, if the common shares sold or exchanged are traded on an established securities market and the U.S. Holder is a cash basis taxpayer or an electing accrual basis taxpayer, which election must be applied consistently from year to year and cannot be changed without the consent of the IRS, the spot exchange rate in effect on the settlement date) and the U.S. Holder's adjusted tax basis in the common shares determined in U.S. dollars. The initial tax basis of the common shares to a U.S. Holder will be the U.S. Holder's U.S. dollar purchase price for the common shares (determined by reference to the spot exchange rate in effect on the date of the purchase, or if the common shares purchased are traded on an established securities market and the U.S. Holder is a cash basis taxpayer or an electing accrual basis taxpayer, which election must be applied consistently from year to year and cannot be changed without the consent of the IRS, the spot exchange rate in effect on the settlement date). An accrual basis U.S. Holder that does not make the special election will recognize exchange gain or loss to the extent attributable to the difference between the exchange rates on the sale date and the settlement date, and such exchange gain or loss generally will constitute ordinary income or loss.

Subject to the discussion under "Passive Foreign Investment Company Rules" above, such gain or loss will be capital gain or loss and will be long-term gain or loss if the common shares have been held for more than one year, subject to the PFIC rules discussed below. Under current law, long-term capital gains of non-corporate U.S. Holders generally are eligible for reduced rates of taxation. The deductibility of capital losses is subject to limitations. Capital gain or loss, if any, recognized by a U.S. Holder generally will be treated as U.S. source income or loss for U.S. foreign tax credit purposes. U.S. Holders are encouraged to consult their own tax advisors regarding the availability of the U.S. foreign tax credit in their particular circumstances.

Medicare Contribution Tax

Certain U.S. Holders that are individuals, estates or certain trusts must pay a 3.8% tax, or "Medicare contribution tax", on their "net investment income." Net investment income generally includes, among other things, dividend income and net gains from the disposition of stock. A U.S. Holder that is an individual, estate or trust should consult its own tax advisor regarding the applicability of the Medicare contribution tax to its income and gains in respect of its investment in our common shares.

Information Reporting and Backup Withholding

Payments of dividends and sales proceeds that are made within the United States or through certain U.S.-related financial intermediaries generally are subject to information reporting, and may be subject to backup withholding, unless (i) the U.S. Holder is a corporation or other exempt recipient or (ii) in the case of backup withholding, the U.S. Holder provides a correct taxpayer identification number and certifies that it is not subject to backup withholding on a duly executed IRS Form W-9 or otherwise establishes an exemption.

Backup withholding is not an additional tax. The amount of any backup withholding from a payment to a U.S. Holder will be allowed as a credit against the U.S. Holder's U.S. federal income tax liability and may entitle the U.S. Holder to a refund, provided that the required information is timely furnished to the IRS.

Certain Reporting Requirements

U.S. Holders paying more than $100,000 for our common shares generally may be required to file IRS Form 926 reporting the payment of the offer price for our common shares to us. Substantial penalties may be imposed upon a U.S. Holder that fails to comply. Each U.S. Holder should consult its own tax advisor as to the possible obligation to file IRS Form 926.

Information with Respect to Foreign Financial Assets

Certain U.S. Holders who are individuals (and, under regulations, certain entities) may be required to report information relating to the common shares, subject to certain exceptions (including an exception for common shares held in accounts maintained by certain U.S. financial institutions), by filing IRS Form 8938 (Statement of Specified Foreign Financial Assets) with their federal income tax return. Such U.S. Holders

who fail to timely furnish the required information may be subject to a penalty. Additionally, if a U.S. Holder does not file the required information, the statute of limitations with respect to tax returns of the U.S. Holder to which the information relates may not close until three years after such information is filed. U.S. Holders should consult their tax advisers regarding their reporting obligations with respect to their ownership and disposition of the common shares.

DESCRIPTION OF SHARE CAPITAL

The following description of our share capital summarizes certain provisions of our articles of incorporation. These summaries do not purport to be complete and are subject to, and qualified in their entirety by reference to, all of the provisions of our articles of incorporation. Moreover, a prospectus supplement relating to a particular offering of our common shares may include terms pertaining to the common shares being offered thereunder that are not within the terms and parameters described in this prospectus.

Our authorized capital consists of an unlimited number of common shares and an unlimited number of preferred shares, issuable in series. As at January 16, 2020, we had 55,378,660 common shares issued and outstanding, all of which are fully paid and non-assessable, and 60,277,193 common shares on a fully diluted basis, including 4,726,943 stock options granted under the stock option plan and 171,590 broker warrants.

Common Shares

Voting Rights. Each of our common shares entitles its holder to notice of, and to one vote at, all meetings of our shareholders. Holders of our common shares are not entitled to cumulative voting.

Dividend Rights. Each of our common shares carries an entitlement to receive dividends if, as and when declared by our board of directors, or the "Board". In the event of the liquidation, dissolution or winding-up of BELLUS Health, our net assets available for distribution to our shareholders will be distributed ratably among the holders of our common shares.

Applicable Limitations on Nonresident or Foreign Owners. There are no applicable limitations on the right of nonresident or foreign owners to hold or vote our common shares imposed by foreign law or by our charter or other constituent documents.

Share Consolidation. On August 15, 2019, we filed articles of amendment for the purpose of effecting a consolidation of our common shares on the basis that each 3.6 outstanding common shares became one post-consolidated common share. No fractional common shares were issued in connection with such consolidation and, in the event that a shareholder would otherwise have been entitled to a fractional common share upon such consolidation, such fractional share was cancelled. Except where otherwise noted, all information in this prospectus and the documents incorporated by reference dated on or after the date of the share consolidation gives effect to such share consolidation.

Preferred Shares

No preferred shares are currently issued; however, they may be issued from time to time in one or more series, the terms of each series, including the number of shares, the designation, rights, preferences, privileges, priorities, restrictions, conditions and limitations, to be determined at the time of creation of each such series by the Board without shareholder approval, provided that all preferred shares will rank, with respect to dividends and return of capital in the event of liquidation, dissolution, winding-up or other distribution of our assets for the purpose of winding-up its affairs, pari passu among themselves and in priority to all common shares or shares of any class ranking junior to the preferred shares. Except as provided for in our articles of incorporation (as amended), the holders of preferred shares shall not be entitled to receive notice of meetings of our shareholders nor to attend thereat and shall not be entitled to vote at any such meeting.

Major Shareholders

As at January 16, 2020, OrbiMed Advisors LLC ("**OrbiMed**"), Power Sustainable Capital Investments Inc. ("**PSCI**"), a subsidiary of Power Corporation of Canada, and Rocabe Investments Inc., a company in which Mr. Roberto Bellini has a 50% equity interest ("**Rocabe**" and, collectively with OrbiMed and PSCI, the "**Major Shareholders**"), together own, directly or indirectly, an aggregate of approximately 29.8% of our outstanding common shares.

BOOK-BASED SYSTEM

Except as otherwise provided in the applicable prospectus supplement, securities will be issued by way of instant deposit under the book-based system administered by CDS Clearing and Depository Services Inc. or a successor (collectively, "**CDS**"), registered in the name of CDS or its nominee. No purchaser of securities will receive a certificate or other instrument from us or CDS evidencing that purchaser's ownership thereof, and no purchaser will be shown on the records maintained by CDS except through a book-entry account of a participant ("**Participant**") in the depository service of CDS acting on behalf of such purchaser. Each purchaser of securities will receive a customer confirmation of purchase from the registered dealer from which the securities are purchased in accordance with the practices and procedures of that registered dealer. The practices of registered dealers may vary, but generally customer confirmations are issued promptly after execution of a customer order. CDS will be responsible for establishing and maintaining book-entry accounts for its Participants having interests in the securities.

Transfer, Conversion, Exchange or Redemption of Securities

Transfer of ownership, conversion, exchange or redemptions of securities will be effected through records maintained by CDS or its nominee for such securities with respect to interests of Participants, and on the records of Participants with respect to interests of persons other than Participants. An owner of a beneficial interest in a security in "book-entry" form who desires to sell or otherwise transfer that interest may do so only through Participants. The ability of that owner to pledge its interest in the security or otherwise take action with respect to its interest in the security may be limited due to the lack of a physical certificate.

Special Situations When Global Security Will be Terminated

If we determine, or CDS notifies us in writing, that CDS is no longer willing or able to discharge properly its responsibilities as depository with respect to the securities and we are unable to locate a qualified successor, or if we at our option elect, or are required by law, to terminate the book-entry system, then the securities will be issued in fully registered form to beneficial owners or their nominees.

TRADING PRICE AND VOLUME OF COMMON SHARES

Information regarding trading price and volume of our issued and outstanding common shares listed on any securities exchange, as applicable, will be provided in each applicable prospectus supplement to this prospectus.

PRIOR SALES

Information regarding prior sales of our common shares will be provided as required in the applicable prospectus supplement.

RISK FACTORS

Investing in our common shares involves a significant amount of risk. You should carefully consider the risks described below, in the applicable prospectus supplement and in the documents incorporated by reference herein and therein before making an investment decision. If any of these risks actually occurs, our business, financial condition, results of operations or prospects could be materially adversely affected. These are not the only risks and uncertainties that we face. Additional risks and uncertainties not presently known to us, or that we currently consider immaterial, may also materially and adversely affect us. In such an event, the trading

price of our common shares could decline and you may lose part or all of your investment in our securities. Any reference in this section to the Company's "products" or "product candidates" includes a reference to BELLUS Health's product candidate and future products or product candidates that may be developed.

This prospectus also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including the risks faced by us described below and elsewhere in this prospectus. See "Forward-Looking Statements" for information relating to these forward-looking statements.

Risks related to our business

We may not be able to maintain our operations and research and development without additional funding, and we may not have access to sufficient capital.

To date, we have financed our operations primarily through public offerings of common shares, private placements, the issuance of convertible notes and research tax credits. We have incurred significant operating losses and negative cash flows from operations since inception. As at September 30, 2019, we had available cash, cash equivalents and short-term investments totaling Cdn$132.2 million. Based on management's estimate and current level of operations, we believe that our current liquidity position is sufficient to finance our operations into the foreseeable future. We will need to raise additional capital to fund our operations and to develop BLU-5937. Our future capital requirements will be substantial and may increase beyond current expectations depending on many factors, such as the duration, scope, rate of progress, results and costs of any preclinical studies and clinical trials for our current or any future product candidates; unexpected delays or developments in seeking regulatory approvals and the outcome thereof; the time and cost in preparing, filing, prosecuting, maintaining, and enforcing patent claims; other unexpected developments encountered in implementing our business development and commercialization strategies; the outcome of any litigation; and arrangements with collaborators. Further, changing circumstances may cause us to consume capital significantly faster than we currently anticipate. We have based the foregoing estimates on assumptions that may prove to be wrong, and we could utilize our available financial resources sooner than we currently expect.

We may seek to raise additional funds through public or private equity or debt financing, collaborations agreements with other companies and/or from other sources. We have no committed source of additional capital and additional funding may not be available on terms that are acceptable to us, or at all. If adequate funding is not available on reasonable terms, we may need to obtain funds on terms less favorable than we would otherwise accept. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of those securities could result in dilution to our shareholders. Moreover, the incurrence of debt financing could result in a substantial portion of our future operating cash flow, if any, being dedicated to the payment of principal and interest on such indebtedness and could impose restrictions on operations. This could render us more vulnerable to competitive pressures and economic downturns. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of BLU-5937 or other future product candidates or other research and development initiatives. We could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

No assurance can be given that any such additional funding will be available or that, if available, it can be obtained on terms favorable to us. The failure to obtain additional financing on favorable terms, or at all, could have a material adverse effect on our business, financial condition, results of operations and prospects.

We have a history of losses and have not generated any product sales revenue to date. We may never achieve or maintain profitability.

Our product candidate, BLU-5937, is still only in development, and as a result, we have not generated any revenues from product sales to date. We have incurred substantial expenses in our efforts to develop BLU-5937, and consequently, have generated operating losses each year since our inception. For the years

ended December 31, 2017 and 2018 and the 9 month period ended September 30, 2019, we incurred net losses of Cdn$1.9 million, Cdn$9.1 million and Cdn$21.3 million, respectively. As of September 30, 2019, we had an accumulated deficit of Cdn$509.4 million. Our losses have adversely affected, and will continue to adversely impact, working capital, total assets, and shareholders' equity. We do not expect to generate any revenues from product sales in the immediate future. We may never successfully commercialize any products. Even if we succeed in developing commercial products, we expect to incur additional operating losses for at least the next several years. If we do not ultimately commercialize products and achieve or maintain profitability, an investment in our shares could result in a significant or total loss.

Our prospects currently depend heavily on the success and market acceptance of BLU-5937, which is still in clinical development.

We currently have no products for sale and may never be able to successfully develop products for sale. We currently believe that our growth and future prospects are mainly dependent on the successful development, regulatory approval and commercialization of our product candidate BLU-5937, which may never occur. We are focusing our efforts and resources into the development of BLU-5937. Our business thus depends on the successful preclinical and clinical development, regulatory approval and commercialization of BLU-5937, for which we must conduct additional preclinical studies and clinical trials, undergo further development activities and seek and receive regulatory approval prior to commercial launch. Further development of BLU-5937 will require substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales, if approved.

We anticipate that our ability to generate revenues will depend on the commercial success of BLU-5937, which will depend upon its market acceptance by purchasers in the pharmaceutical market and the future market demand and medical need for products and research utilizing BLU-5937. Most prescription drug candidates never reach the clinical development stage and even those that do reach clinical development have only a small chance of successfully completing clinical development and gaining regulatory approval. If we are unable to successfully commercialize BLU-5937, we may never generate revenues. There is also the risk that the actual market size or opportunity for BLU-5937 is not certain. If BLU-5937 reaches commercialization and there is low market demand for BLU-5937 or the market for BLU-5937 develops less rapidly than we anticipate, we may not have the ability to shift our resources to the development of alternative products. Failure to gain market acceptance of BLU-5937 or an incorrect estimate in the nature and size of our market could have a material adverse effect on us.

We rely on third parties to conduct preclinical studies and clinical trials for BLU-5937, and if they do not properly and successfully perform their obligations to us, we may not be able to obtain regulatory approvals for BLU-5937.

We have designed the clinical trials for BLU-5937. However, we rely on contract research organizations and other third parties to assist in managing, monitoring and otherwise carrying out these trials. We compete with many other companies for the resources of these third parties. The third parties on whom we rely generally may terminate their engagements at any time, and having to enter into alternative arrangements would delay development and commercialization of our product candidate. The U.S. Food and Drug Administration, or the "**FDA**", and comparable foreign regulatory authorities require compliance with regulations and standards for designing, conducting, monitoring, recording, analyzing, and reporting the results of clinical trials to assure that the data and results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Although we rely on third parties to conduct our clinical trials, they are not our employees, and we are responsible for ensuring that each of these clinical trials is conducted in accordance with our general investigational plan, protocol and other requirements. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities.

If these third parties do not successfully carry out their duties under their agreements, if the quality or accuracy of the data they obtain is compromised due to their failure to adhere to clinical trial protocols or to regulatory requirements, or if they otherwise fail to comply with clinical trial protocols or meet expected deadlines, the clinical trials of BLU-5937 may not meet regulatory requirements. If clinical trials do not

meet regulatory requirements or if these third parties need to be replaced, preclinical development activities or clinical trials may be extended, delayed, suspended or terminated. If any of these events occur, we may not be able to obtain regulatory approval of BLU-5937 on a timely basis or at all.

We rely completely on one third-party contract manufacturer to manufacture the active pharmaceutical ingredient, or "API", for BLU-5937 and another third-party contract manufacturer to manufacture the final drug product, and we intend to rely on third parties to produce non-clinical, clinical and commercial supplies of BLU-5937 and any other future product candidates.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to internally manufacture our clinical drug supply of BLU-5937, or any other product candidates we may develop in the future, for use in the conduct of our research and development activities, preclinical studies and clinical trials, and we lack the internal resources and the capability to manufacture any product candidates on a clinical or commercial scale. We currently have the API for BLU-5937 manufactured by one third-party contract manufacturer and final drug product supplied by another contract manufacturer, and do not currently have backup manufacturing capacity.

We plan to continue to rely on contract manufacturers for the foreseeable future to produce quantities of products and substances necessary for research and development, preclinical studies, human clinical trials and product commercialization, and to perform their obligations in a timely manner and in accordance with applicable government regulations. While we intend to contract for the commercial manufacture of our product candidates, we may not be able to identify and qualify contractors or obtain favorable contracting terms.

If our current or future third-party manufacturers do not perform as agreed, or breach or terminate their agreements with us, significant additional time and costs would be required to effect a transition to a new contract manufacturer. If we are unable to retain our current contractors, or are unable to secure arrangements with new contractors to provide manufacturing services in a timely manner and on acceptable terms as needed, it will delay or prevent the development, promotion, marketing, or sale of BLU-5937, if approved, or any other future product candidates we may develop, and have a negative effect on our operations and financial condition. Moreover, if a replacement to our current or future contract manufacturers is required, the ability to establish second-sourcing or find a replacement manufacturer may be difficult due to the lead times generally required to manufacture drug products and the need for regulatory compliance inspections and approvals of any replacement manufacturer, all of which factors could result in production delays and additional costs.

Manufacturing of API and final drug products is complex and requires significant expertise. Difficulties could be encountered in production, particularly in scaling up and validating production. There can be no assurance that contract manufacturers will be successful at scaling up and producing BLU-5937 with the required quality and in the quantities and timelines that will be needed for clinical and/or commercial purposes. So far, we have only produced small quantities of BLU-5937 at kilogram scale for use in preclinical studies and clinical trials.

Our reliance on these contract manufacturers also exposes us to the possibility that they, or third parties with access to their facilities, will have access to and may appropriate our trade secrets or other proprietary information.

We rely on third-party contract manufacturers that are located outside of Canada. As a result, our operations are subject to customary risks related to the import of goods, including fluctuations in the value of currencies, changes in import duties, exchange controls, trade restrictions, work stoppages and general political and economic conditions in foreign countries. The countries from which we import pharmaceutical ingredients may, from time to time, impose new duties, tariff's or other restrictions or adjust presently prevailing duties or tariffs, which could adversely impact our ability to purchase such pharmaceutical ingredients or significantly increase the cost of doing so. The occurrence of any of these risks could delay or prevent the development, promotion, marketing, or sale of BLU-5937, if approved, or of any other future product candidates we may develop, and have a negative effect on our operations and financial condition.

The clinical effectiveness of BLU-5937 is not yet supported by clinical data.

The preclinical toxicology studies and the Phase 1 topline data announced in November 2018 showed that BLU- 5937 has a favorable safety and tolerability profile. However, the clinical safety of BLU-5937 has to be demonstrated through further clinical studies. The clinical effectiveness of BLU-5937 is not yet supported by clinical data and the medical community has not yet developed a large body of peer reviewed literature that supports the safety and efficacy of BLU-5937. If future studies call into question the safety or efficacy of BLU-5937 or any other product candidates we may develop in the future, our business, financial condition, results of operations or prospects could be adversely affected.

Even if BLU-5937 or any other product candidates we may develop in the future successfully complete the clinical trials and receive the regulatory approval necessary to market the product candidates to the public, there is also the risk of unknown side effects, which may not appear until the product candidates are on the market and may result in delay or denial of regulatory approval or withdrawal of previous approvals, product recalls or other adverse events, which could materially adversely affect us.

Our clinical trials may not yield results that will enable us to obtain regulatory approval for our current or future product candidates.

We will only receive regulatory approval for a product candidate if we can demonstrate in carefully designed and conducted clinical trials that the product candidate is safe and effective. We do not know whether our current or any future clinical trials will demonstrate sufficient safety and efficacy to obtain the requisite regulatory approvals or if they will result in marketable products.

Clinical trials are lengthy, complex, costly, and uncertain processes. It takes several years to complete testing, and failure can occur at any stage of testing. The early stage of our product candidate involves risks related to safety, efficacy, drug metabolism, pharmacokinetic profile, tolerability, manufacturing, formulation and distribution, among others. Results attained in preclinical testing and early clinical studies or trials may not be indicative of results that are obtained in later studies. We have suffered, and may suffer further, significant setbacks in advanced clinical trials, even after promising results in earlier studies. For instance, in June 2016, we announced that KIACTA (eprodisate) did not meet the primary efficacy endpoint in a Phase 3 clinical trial. Based on results at any stage of clinical trials, we may decide to repeat or redesign a trial or discontinue the development of a product candidate. Furthermore, actual results may vary once the final and quality-controlled verification of data and analyses has been completed. If we fail to adequately demonstrate the safety and efficacy of BLU-5937, we will not be able to obtain the required regulatory approvals to commercialize that product candidate.

Clinical trials are subject to continuing oversight by governmental regulatory authorities and institutional review boards, and must meet the requirements of these authorities; must meet requirements for informed consent; and must meet requirements for good clinical practices.

We may not be able to comply with these requirements. We rely on third parties, including contract research organizations and outside consultants, to assist in managing and monitoring clinical trials. Our reliance on these third parties may result in delays in completing, or in failing to complete, these trials if one or more third parties fail to perform with the speed and level of competence expected. If clinical trials for a product candidate are unsuccessful, we will be unable to commercialize such product candidate. If one or more of the clinical trials is delayed, we will be unable to meet our anticipated development or commercialization timelines. Either circumstance could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we encounter difficulties enrolling patients in clinical trials, the trials could be delayed or otherwise adversely affected.

Clinical trials for product candidates require us or third parties we contract with to identify and enroll a large number of patients with the disorder under investigation. We or the third parties we contract with may not be able to enroll a sufficient number of patients to complete clinical trials in a timely manner. Patient enrollment is a function of many factors, including the following: design of the protocol, size of the patient population, eligibility criteria for the trial in question, perceived risks and benefits of the drug under

study, availability of competing therapies, efforts to facilitate timely enrollment in clinical trials, patient referral practices of physicians, and availability of clinical trial sites. If we or the third parties we contract with have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay or terminate ongoing clinical trials.

The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of the success of later-stage clinical trials.

The outcome of preclinical testing and earlier-stage clinical trials may not be predictive of the success of later-stage clinical trials. BLU-5937 and any other product candidates we may develop may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Numerous companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials, and we cannot be certain that we will not face similar setbacks. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Furthermore, the failure of any product candidate to demonstrate safety and efficacy in any clinical trial could negatively impact the perception of any other product candidates then under development and/or cause applicable regulatory authorities to require additional testing before approving any other product candidates.

Interim topline and preliminary results from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures, which could result in material changes in the final data.

From time to time, we may publish interim topline or preliminary results from our clinical trials. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common shares to fluctuate significantly.

Even if we or any future partners obtain regulatory approvals for our product candidates, we will be subject to ongoing government regulation.

Even if regulatory authorities approve BLU-5937 or any future product candidate we may develop, the manufacturing, marketing, and sale of such products will be subject to strict and ongoing regulation. Compliance with such regulation may be costly and consume substantial financial and management resources. For example, an approval for a product may be conditioned on conducting costly post-marketing follow-up studies. In addition, if, based on these studies, a regulatory authority does not believe that the drug demonstrates a benefit to patients, such authority could limit the indications for which the product may be sold or revoke the product's regulatory approval.

We and our contract manufacturers are required to comply with applicable current Good Manufacturing Practice regulations for the manufacture of product candidates. These regulations include requirements relating to quality assurance, as well as the corresponding maintenance of records and documentation. Manufacturing facilities must be approved before they can be used in the commercial manufacturing of products and are subject to subsequent periodic inspection by regulatory authorities. In addition, material changes in the methods of manufacturing or changes in the suppliers of raw materials are subject to further regulatory review and approval.

If we or any future marketing collaborators or contract manufacturers fail to comply with applicable regulatory requirements, we may be subject to sanctions, including fines, drug recalls or seizures, injunctions, total or partial suspension of production, civil penalties, withdrawals of previously granted regulatory approvals, and criminal prosecution. Any of these penalties could delay or prevent the promotion, marketing, or sale of our products.

In addition, we are currently or will in the future be subject to healthcare regulation and enforcement by the federal government and the states in which we will conduct our business once our product candidates are approved by the FDA and commercialized in the United States. In addition to the FDA's restrictions on marketing of pharmaceutical products, the healthcare laws and regulations that may affect our ability to operate include: the federal fraud and abuse laws, including the federal anti-kickback and false claims laws; federal data privacy and security laws; and federal transparency laws related to payments and/or other transfers of value made to physicians and other healthcare professionals and teaching hospitals. Many states have similar laws and regulations that may differ from each other and federal law in significant ways, thus complicating compliance efforts. These laws may adversely affect our sales, marketing and other activities with respect to any product candidate for which we receive approval to market in the United States by imposing administrative and compliance burdens on us.

Because of the breadth of these laws and the narrowness of available statutory exceptions and regulatory safe harbors, it is possible that some of our business activities, particularly any sales and marketing activities after a product candidate has been approved for marketing in the United States, could be subject to legal challenge and enforcement actions. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to significant civil, criminal, and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We may not achieve our projected development goals in the announced and expected time frames.

From time to time, we set goals for and make public statements regarding the expectations for and timing of the accomplishment of objectives material to our success, such as the commencement and completion of clinical trials, expected results, anticipated regulatory submission and approval dates, and timing of product launch. The actual timing of these events can vary dramatically due to factors such as delays or failures in clinical trials, the uncertainties inherent in the regulatory approval process, and delays in achieving manufacturing or marketing arrangements sufficient to commercialize products. There can be no assurance that our clinical trials will be completed, that we will make regulatory submissions or receive regulatory approvals as planned, or that we will be able to adhere to our current schedule for the launch of BLU-5937 or any other future product candidates we may develop. If we fail to achieve one or more of these milestones as planned, the price of our common shares would likely be adversely affected.

If we or our partners fail to obtain acceptable prices, coverage or adequate reimbursement for our products, our ability to generate revenues will be diminished.

Patients in the United States and elsewhere generally rely on third-party payors to reimburse part or all of the costs associated with their prescription drugs. Accordingly, our ability to successfully commercialize our products would depend significantly on the ability to obtain acceptable prices and the availability of coverage and adequate reimbursement from third-party payors, such as government and private insurance plans. Coverage and reimbursement policies for drug products can differ significantly among payors as there is no uniform policy of coverage and reimbursement for drug products among U.S. third-party payors. There may be significant delays in obtaining coverage and reimbursement as the process of determining coverage and reimbursement is often time-consuming and costly which will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage or adequate reimbursement will be obtained. While we have not commenced discussions with any such parties, these third-party payors frequently require companies to provide predetermined discounts from list prices, and they are increasingly challenging the prices charged for pharmaceuticals and other medical products. Our products may not be considered cost-effective, and reimbursement to the patient may not be available or sufficient to allow us to sell our products on a

competitive basis. Even if we obtain coverage for a given product candidate, the associated reimbursement rate may not be adequate to cover our costs, including research, development, intellectual property, manufacture, sale and distribution expenses, or may require co-payments that patients find unacceptably high.

In addition, the continuing efforts of third-party payors to contain or reduce the costs of healthcare through various means may limit our commercial opportunity and reduce any associated revenue and profits. We expect proposals to implement similar government controls to continue. In addition, increasing emphasis on managed care will continue to put pressure on the pricing of pharmaceutical and biopharmaceutical products. Cost-control initiatives could decrease the price that we or any current or potential collaborators could receive for any of the products and could adversely affect profitability. In addition, in Canada and in many other countries, where significant healthcare reforms are currently under discussion, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to government control. In the United States, there have been and continue to be a number of healthcare-related legislative initiatives that have significantly affected the pharmaceutical industry. For example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the Affordable Care Act, was passed in March 2010, and substantially changed the way healthcare is financed by both governmental and private insurers, and continues to significantly impact the pharmaceutical industry. There also has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. If we fail to obtain acceptable prices, coverages or an adequate level of reimbursement for our products, the sales of the products would be adversely affected or there may be no commercially viable market for our products.

Competition in the biopharmaceutical industry is intense, and development by other companies could render our product candidate or any future product candidates or technologies non-competitive.

The biopharmaceutical industry is intensely competitive and is subject to rapid and significant change. We face potential competition from many sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies. We consider our primary competitors to be those companies that are developing products specifically to treat chronic cough and those companies that develop products that, when approved, could be used off-label to treat cough. We are aware of other companies targeting chronic cough as the primary outcome measure in clinical studies of products. There are multiple companies developing products at varying stages of development specifically intended to treat chronic cough including Merck & Co., Bayer AG, Shionogi Inc. and NeRRe Therapeutics Ltd, some of which have substantially greater product development capabilities and financial, scientific, marketing, and human resources than us. Of these companies, Merck, Bayer and Shionogi are developing P2X3 antagonists for chronic cough that could compete directly with BLU-5937. Moreover, there are multiple companies developing therapeutic treatments for atopic dermatitis specifically, or various other forms of pruritus which could also have a therapeutic effect on atopic dermatitis itch including Sanofi S.A., Bayer AG, Pfizer Inc., Novartis International AG, LEO Pharma Inc., Menlo Therapeutics Inc., Vanda Pharmaceuticals Inc., Trevi Therapeutics Inc., Galderma S.A., Sienna Biopharmaceuticals, Inc., Tioga Pharmaceuticals, Inc. and Cara Therapeutics Inc.

We are heavily dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we would not be able to continue developing or commercializing BLU-5937. If we breach any of the agreements under which we license the use, development and commercialization rights to BLU-5937 or any other future product candidate or technology from third parties or if certain insolvency events were to occur, we could lose license rights that are critical to our business.

We have an exclusive worldwide license to develop and commercialize BLU-5937 pursuant to a license agreement with the NEOMED Institute, now adMare Bionnovations ("**NEOMED**"), that is critical to our business, which is subject to termination for breach of our terms and, therefore, our rights may only be available to us for as long as our development and commercialization activities are sufficient to meet the

terms of the license. In addition, we may need to enter into additional license agreements in the future. Our existing license agreements impose, and any future license agreements may impose on us, various developments, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license, which would have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, our current or future licenses may provide for a reversion to the licensor of our rights in regulatory filings or other intellectual property or data that we regard as our own in the event the license terminates under certain circumstances, such as due to breach.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including with respect to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the rights of our licensors under the license agreements; and
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of BLU-5937 and any future product candidates, and what activities satisfy those diligence obligations.

Any disputes with our licensors over intellectual property that we have licensed from them may prevent or impair our ability to maintain our current licensing arrangements on acceptable terms. Termination or expiry of our license agreements could result in the loss of significant rights and could materially harm our ability to further develop and commercialize BLU-5937 or other future product candidates.

We depend on our licensors to protect a significant portion of our proprietary rights that derive from license agreements, including our exclusive worldwide license with NEOMED to develop and commercialize BLU-5937. BLU-5937 is covered by a patent that is not owned by us but is instead licensed to us by NEOMED. Moreover, our licensors under current licenses retain and our licensors under future licenses may retain certain rights and obligations.

Our business could suffer, for example, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

We may not obtain adequate protection for our products through our intellectual property.

Our success depends, in large part, on our ability to protect our competitive position through patents, trade secrets, trademarks, and other intellectual property rights. Our success, competitive position and future revenues with respect to these product candidates will depend, in part, on our ability to protect our intellectual property. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our proprietary rights are covered by valid and enforceable patents or are effectively maintained as trade secrets. We attempt to protect our proprietary position by maintaining trade secrets and by filing U.S. and foreign patent applications related to our in-licensed technology, inventions and improvements that are important to the development of our business. Our failure to do so may adversely affect our business and competitive position.

The patent positions of pharmaceutical and biopharmaceutical firms, including ours, are uncertain and involve complex questions of law and fact for which important legal issues remain unresolved. The patents issued or to be issued to us may not provide us with any competitive advantage. We may not be able to protect our intellectual property rights throughout the world. Our patents may be challenged by third parties in patent litigation. In addition, it is possible that third parties with drugs that are very similar to ours will circumvent our patents by means of alternate designs or processes. We may have to rely on method of use protection for our compounds in development and any resulting drugs, which may not confer the same level of protection as protection of our compounds per se. We may be required to disclaim part of the term of certain patents. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that our patents would, if challenged, be held by a court to be valid or enforceable or that a competitor's technology or drug would be found by a court to infringe our patents.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time. Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Patent applications relating to or affecting our business may have been filed by a number of pharmaceutical and biopharmaceutical companies and academic institutions. A number of the technologies in these applications or patents may conflict with our technologies, patents, or patent applications, and such conflict could reduce the scope of patent protection that we could otherwise obtain. We could become involved in interference proceedings in the United States in connection with one or more of our patents or patent applications to determine priority of invention. Our granted patents could also be challenged and revoked in opposition proceedings in certain countries outside of the United States. In addition to patents, we rely on trade secrets and proprietary know-how to protect our intellectual property. We generally require employees, consultants, outside scientific collaborators, and sponsored researchers and other advisors to enter into confidentiality agreements. These agreements provide that all confidential information developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all of the technology that is conceived by the individual during the course of employment is our exclusive property. These agreements may not provide meaningful protection or adequate remedies in the event of unauthorized use or disclosure of proprietary information. In addition, it is possible that third parties could independently develop proprietary information and techniques substantially similar to ours or otherwise gain access to our trade secrets.

We may obtain the right to use certain technology under license agreements with third parties. Our failure to comply with the requirements of material license agreements could result in the termination of such agreements, which could cause us to terminate the related development program and cause a complete loss of investment in that program. As a result of the foregoing factors, we may not be able to rely on our intellectual property to protect our products in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

We seek to protect our confidential proprietary information, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and we cannot be certain

that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. We also seek to preserve the integrity and confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our information technology systems, but it is possible that these security measures could be breached. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

We may infringe the intellectual property rights of others.

Our commercial success depends significantly on our ability to operate without infringing on the patents and other intellectual property rights of third parties. There could be issued patents of which we are not aware that our products infringe or patents that we believe we do not infringe, but that we may ultimately be found to infringe. Moreover, patent applications are, in some cases, maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our products infringe. For example, pending applications may exist that provide support or can be amended to provide support for a claim that results in an issued patent that our drug infringes.

The biopharmaceutical industry has produced a proliferation of patents, and it is not always clear to industry participants which patents cover various types of products. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. We believe that BLU-5937 does not infringe any valid claim of these patents, although there can be no assurances of this. In the event of an infringement or violation of another party's patent, we may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost. Any inability to secure licenses or alternative technology could result in delays in the introduction of drugs or lead to prohibition of the manufacture or sale of drugs by us.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Third parties may assert patent or other intellectual property infringement claims against us or our other licensors arising from the manufacture, use, or sale of our current or future product candidates. An unfavorable outcome could result in loss of patent rights and require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may become involved in lawsuits or other proceedings to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents or other intellectual property. If we were to initiate legal proceedings against a third party to enforce a patent covering our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent

withheld relevant information from the United States Patent and Trademark Office, or "**USPTO**", or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. The validity of our current or future patents or patent applications or those of our licensors may also be challenged in interference or derivation proceedings, opposition, post grant review, inter partes review, or other similar enforcement and revocation proceedings, provoked by third parties or brought by us. Our patents could be found invalid, unenforceable, or their scope significantly reduced.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or other proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

Patent litigation is costly and time consuming and may subject us to liabilities.

Our involvement in any patent litigation, interference, post-grant proceedings such as inter partes review or opposition, or other administrative proceedings will likely cause us to incur substantial expenses, and the efforts of technical and management personnel will be significantly diverted. In addition, an adverse determination in litigation could subject us to significant liabilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common shares.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other

proprietary information, of any of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

The market price of our common shares experiences a high level of volatility due to factors such as the volatility in the market for biotechnology stocks generally and the short-term effect of a number of possible events.

We are a public growth company in the biotechnology sector. As frequently occurs among these companies, the market price for our common shares may experience a high level of volatility. During the 12-month period ended on the date of this prospectus, giving effect to the one-for-3.6 consolidation of our common shares effected on August 15, 2019, our common shares traded between Cdn$3.49 and Cdn$12.38 per share on the TSX. Since their initial NASDAQ listing on September 9, 2019, our common shares traded between US$5.55 and US$9.04 per share on NASDAQ.

Numerous factors, including many over which we have no control, may have a significant impact on the market price of our common shares, including, among other things, the following: (1) clinical and regulatory developments regarding our product candidate and those of our competitors; (2) arrangements or strategic partnerships by our competitors; (3) other announcements by us or our competitors regarding technological, drug development, sales, or other matters; (4) patent or other intellectual property achievements or adverse developments; (5) arrivals or departures of key personnel; (6) changes in financial estimates and recommendations by securities analysts; (7) government regulatory action affecting our product candidate and our competitors' products in the United States, Canada, and foreign countries; (8) actual or anticipated fluctuations in revenues or expenses; (9) general market conditions and fluctuations for the emerging growth and biopharmaceutical market sectors; (10) failure to enter into favorable third-party manufacturing agreements; (11) events related to threatened, new, or existing litigation; (12) economic conditions in the United States, Canada, or abroad; (13) purchases or sales of blocks of our securities; and (14) difficulties in our ability to obtain additional financing.

The recent listing of our common shares on NASDAQ may increase share price volatility due to various factors, including that the stock market in recent years has experienced extreme price and trading volume fluctuations that often have been unrelated or disproportionate to the operating performance of individual companies. These broad market fluctuations may adversely affect the price of our common shares, regardless of our operating performance. In addition, sales of substantial amounts of our common shares in the public market after any offering, or the perception that those sales may occur, could cause the market price of our common shares to be adversely affected.

As at January 16, 2020, our Major Shareholders together own, directly or indirectly, an aggregate of approximately 29.8% of our outstanding common shares. A decision by one or more of our Major Shareholders or any other significant shareholder to sell a substantial amount of our common shares could

cause the trading price of our common shares to be adversely affected. Furthermore, shareholders may initiate securities class action lawsuits if the market price of our common shares drops significantly, which may cause us to incur substantial costs and could divert the time and attention of our management.

These factors, among others, could depress the trading price of our securities. Because we may experience high volatility in our common shares, individuals or entities should not invest in our common shares unless prepared to absorb a significant loss of capital. At any given time, investors may not be able to sell their shares at a price that is acceptable or at all. The market liquidity for our stock is low. While a more active trading market may develop in the future, the limited market liquidity for our common shares may affect an investor's ability to sell at a price that is satisfactory to them or at all.

We do not expect to pay any cash dividends for the foreseeable future.

Investors should not rely on an investment in our common shares to provide dividend income. We do not anticipate that we will pay any cash dividends to holders of our common shares in the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our operations. In addition, any future debt financing arrangement may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common shares. Accordingly, investors must rely on sales of their common shares after price appreciation, which may never occur, as the only way to realize any return on their investment. As a result, investors seeking cash dividends should not purchase our common shares.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common shares will depend, in part, on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover our company downgrade our common shares or publish inaccurate or unfavorable research about our business, our share price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our share price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our common shares could decrease, which might cause our share price and trading volume to decline.

We would not be able to successfully commercialize product candidates if we are unable to create sales, marketing, and distribution capabilities or make adequate arrangements with third parties, including entering into collaborations with partners, for such purposes.

In order to commercialize our product candidates successfully, we could, on a product-by-product basis, either develop internal sales, marketing, and distribution capabilities or make arrangements with third parties, including entering into collaborations with partners, to perform some or all of these services. We currently have no marketing capabilities and sales force. To the extent that we internally develop a sales force, the cost of establishing and maintaining a sales force would be substantial and may exceed our cost effectiveness. In addition, in marketing our drugs, we would likely compete with many companies that currently have extensive and well-funded marketing and sales operations. Despite marketing and sales efforts, we may be unable to compete successfully against these companies. We may not be able to do so on favorable terms. We could rely on third parties to market and sell our products in certain territories, rather than establishing an internal sales force. When we contract with third parties, including entering into collaborations with partners, for the sale and marketing of our products, revenues depend upon the efforts of these third parties, which may not be successful. If we fail to establish successful marketing and sales capabilities or to make arrangements with third parties for such purposes, our business, financial condition, results of operations and prospects will be materially adversely affected.

We are subject to intense competition for skilled personnel. The loss of key personnel or the inability to attract additional personnel could impair our ability to conduct operations.

We are highly dependent on our management and staff; the loss of whose services might adversely impact our ability to achieve our objectives. Recruiting and retaining qualified management and other personnel is critical to our success. Competition for skilled personnel is intense, and the ability to attract and retain qualified personnel may be affected by such competition. We do not maintain "key person" insurance for any of our key personnel.

We are subject to the risk of product liability claims, for which we may not have, or may not be able to obtain, adequate insurance coverage.

Human therapeutic products involve the risk of product liability claims and associated adverse publicity. Currently, our principal risks relate to participants in the clinical trials who may suffer unintended consequences. Claims might be made directly by consumers, patients, healthcare providers, or pharmaceutical companies or others selling or consuming any of our products, if approved. We may not have or be able to obtain or maintain sufficient and affordable insurance coverage, including coverage for potentially very significant legal expenses. Without sufficient coverage, any claim brought against us could have a materially adverse effect on our business, financial condition, results of operations or prospects.

Legislative actions, potential new accounting pronouncements, and higher insurance costs are likely to impact our future financial position or results of operations.

Future changes in financial accounting standards may cause adverse, unexpected revenue or expense fluctuations and affect our financial position or results of operations. New pronouncements and varying interpretations of pronouncements have occurred with greater frequency and are expected to occur in the future, and we may make, or may be required to make, changes in our accounting policies in the future. Compliance with changing regulations of corporate governance and public disclosure, notably with respect to internal controls over financial reporting, may result in additional expenses. Changing laws, regulations, and standards relating to corporate governance and public disclosure are creating uncertainty for companies like us, and insurance costs are increasing as a result of this uncertainty.

We may incur losses associated with foreign currency fluctuations.

Our functional and reporting currency is the Canadian dollar. Our operations are, in some instances, conducted in currencies other than the Canadian dollar (principally in U.S. dollars) and a portion of our net monetary assets is denominated in other currencies (principally in U.S. dollars). Fluctuations in the value of foreign currencies relative to the Canadian dollar could cause us to incur currency exchange losses.

We may incur losses due to adverse decisions by tax authorities.

Our income tax reporting is subject to audit by tax authorities. The effective tax rate may change from year to year based on the mix of income; non-deductible expenses; changes in tax law; and changes in the estimated values of future income tax assets and liabilities.

We may enter into transactions and arrangements in the ordinary course of business in which the tax treatment is not entirely certain. We must therefore make estimates and judgments in determining our consolidated tax provision. In addition, we apply for numerous tax credits that play an important role in our financial planning and we are not certain that the tax authorities will grant them. The final outcome of any audits by taxation authorities may differ from estimates and assumptions used in determining the consolidated tax provisions and accruals. This could result in a material effect on our consolidated research tax credits, income tax provision, financial position and the net income/loss for the period in which such determinations are made.

We are subject to taxation in Canada and were subject to taxation in certain foreign jurisdictions prior to the corporate reorganization. Our effective tax rate and tax liability are determined by a number of factors, including the amount of taxable income in particular jurisdictions, the tax rates in these jurisdictions, tax treaties between jurisdictions, the extent to which we transfer funds to and repatriate funds from our subsidiaries and future changes in laws. An adverse interpretation or ruling by one of the taxing authorities in a jurisdiction in which we operate or a change in law could increase our tax liability or result in the imposition of penalty payments, which could adversely impact our operating results.

Our Major Shareholders have influence over our business and corporate matters, including those requiring shareholder approval. This could delay or prevent a change in control. Sales of common shares by our largest shareholders could have an impact on the market price of our common shares.

Our Major Shareholders together own, directly or indirectly, an aggregate of approximately 29.8% of our outstanding common shares as at January 16, 2020. Pursuant to a board representation agreement dated December 18, 2018 between us and Orbimed, Orbimed is entitled to cause one nominee to be

included in the list of management nominees to be proposed for election to our Board at each shareholders' meeting occurring following that date. Orbimed's nomination right terminates on the date Orbimed ceases to beneficially hold at least 10% of our issued and outstanding common shares. OrbiMed's nominated candidate is Mr. Khuong. In addition, pursuant to board representation agreements dated April 16, 2009, between us and each of PSCI and a predecessor to Rocabe (the "**2009 Board Representation Agreements**"), each of PSCI and Rocabe is entitled to cause two nominees to be included in the list of management nominees to be proposed for election to the Board at each shareholders meeting occurring following that date. Despite their rights, each of PSCI and Rocabe has only nominated one candidate. PSCI's and Rocabe's right to two nominees each shall terminate on the date each of PSCI, on the one hand, and Rocabe, the FMRC Family Trust ("**FMRC**") and 1324286 Alberta Limited, a wholly-owned subsidiary of FMRC, collectively, on the other hand, ceases to beneficially hold at least 7.5% of our issued and outstanding common shares. Therefore, OrbiMed, PSCI, FMRC, Rocabe and certain persons related to such entities have the ability to exercise a significant degree of influence over our business and the outcome of various corporate matters, including those requiring shareholder approval. In particular, this concentration of ownership may have the effect of delaying or deferring a change in control of the Company and may adversely affect the price of our common shares.

We may be required to make a payment under an indemnity agreement.

In March 2017, we entered into a share purchase agreement with Taro for the sale of our wholly-owned subsidiary Thallion Pharmaceuticals Inc., including all the rights to the product candidate Shigamab™. We agreed to indemnify Taro, subject to certain conditions and limitations, for losses which it may suffer or incur, arising out of any debts, liabilities, commitments or obligations of any nature resulting from any matters, actions, events, facts or circumstances related to the activities or affairs of Thallion, which occurred prior to the effective time of the share purchase agreement. We have no indemnity provision recorded as at September 30, 2019.

If we are a passive foreign investment company, or PFIC, for U.S. federal income tax purposes, the consequences to U.S. holders of our common shares may be adverse.

Under the U.S. Internal Revenue Code of 1986, as amended, or the "**Code**", we will be classified as a PFIC in respect of any taxable year in which either (i) 75% or more of our gross income consists of certain types of "passive income" or (ii) 50% or more of the average quarterly value of our assets is attributable to "passive assets" (assets that produce or are held for the production of passive income). For purposes of these tests, passive income includes dividends, interest, gains from the sale or exchange of investment property and certain rents and royalties. In addition, for purposes of the above calculations, if we directly or indirectly own at least 25% by value of the shares of another corporation, we will be treated as if it held our proportionate share of the assets and received directly our proportionate share of the income of such other corporation. PFIC status is a factual determination that needs to be made annually after the close of each taxable year, on the basis of the composition of our income, the relative value of our active and passive assets, and our market capitalization. For this purpose, our PFIC status depends in part on the application of complex rules, which may be subject to differing interpretations, relating to the classification of our income and assets. Based on our interpretation of the law, our recent financial statements, and taking into account expectations about our income, assets and activities, we believe that we were a PFIC for the taxable year ended December 31, 2018 and expect that we will be a PFIC for the current taxable year.

If we are a PFIC for any taxable year during which a U.S. Holder (as defined below under "Material United States Federal Income Tax Considerations for U.S. Holders") holds our common shares, we will continue to be treated as a PFIC with respect to such U.S. Holder in all succeeding years during which the U.S. Holder owns the common shares, regardless of whether we continue to meet the PFIC test described above, unless the U.S. Holder makes a specified election once we cease to be a PFIC. If we are classified as a PFIC for any taxable year during which a U.S. Holder holds our common shares, the U.S. Holder may be subject to adverse tax consequences regardless of whether we continue to qualify as a PFIC, including ineligibility for any preferred tax rates on capital gains or on actual or deemed dividends, interest charges on

certain taxes treated as deferred, and additional reporting requirements. In certain circumstances, a U.S. Holder may alleviate some of the adverse tax consequences attributable to PFIC status by making either a "qualified electing fund," or "QEF", election or a mark-to-market election (if our common shares constitute "marketable" securities under the Code.

For further discussion of the PFIC rules and the adverse U.S. federal income tax consequences in the event we are classified as a PFIC, see the section of this prospectus entitled "Material United States Federal Income Tax Considerations for U.S. Holders."

We are an emerging growth company and intend to take advantage of reduced disclosure requirements applicable to emerging growth companies, which could make our common shares less attractive to investors.

We are an "emerging growth company" as defined in the JOBS Act. We will remain an emerging growth company until the earliest to occur of (i) the last day of the fiscal year in which we have total annual gross revenue of US$1.07 billion or more; (ii) December 31, 2024 (the last day of the fiscal year ending after the fifth anniversary of the date of the completion of the first sales of its common equity pursuant to an effective registration statement under the U.S. Securities Act; (iii) the date on which we have issued more than US$1 billion in non-convertible debt securities during the prior three-year period; or (iv) the date we qualify as a "large accelerated filer" under the rules of the SEC, which means the market value of our common shares held by non-affiliates exceeds US$700 million as of the last business day of its most recently completed second fiscal quarter after we have been a reporting company in the United States for at least 12 months. For so long as we remain an emerging growth company, we are permitted to and intend to rely upon exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 ("**Section 404**") of the Sarbanes-Oxley Act Sarbanes-Oxley Act (2002), as amended (the "**Sarbanes-Oxley Act**").

We may take advantage of some, but not all, of the available exemptions available to emerging growth companies. We cannot predict whether investors will find our common shares less attractive if we rely on these exemptions. If some investors find our common shares less attractive as a result, there may be a less active trading market for our common shares and our share price may be more volatile.

Brexit may create volatility in markets and uncertainty regarding future laws and regulations in the United Kingdom and the rest of Europe.

Our Phase 2 clinical trial is being conducted at 12 clinical sites located in the United Kingdom and United States. In June 2016, a majority of voters in the United Kingdom elected to withdraw from the European Union in a national referendum. While the terms of any withdrawal are subject to an ongoing negotiation period, the referendum has led to volatility in the financial markets of the United Kingdom and more broadly across Europe and may lead to a weakening in consumer, corporate and financial confidence in such markets. The referendum has also created significant uncertainty about the future relationship between the United Kingdom and the European Union, including with respect to the laws and regulations that will apply as the United Kingdom determines which European Union laws to replace or replicate in the event of a withdrawal, and has also given rise to calls for the governments of other European Union member states to consider withdrawal. The risks of changing laws and regulations in the United Kingdom are creating uncertainty for companies like us. Compliance with any such changing laws and regulations may be costly and consume substantial financial and management resources, as well as delay or prevent the development, promotion, marketing, or sale of our product candidates. The extent and process by which the United Kingdom may exit the European Union, and the longer term economic, legal, political and social framework to be put in place between the United Kingdom and the European Union are likely to lead to ongoing political and economic uncertainty and periods of exacerbated volatility in both the United Kingdom and in wider European markets for some time. This mid-to-long-term uncertainty may have an adverse effect on global economic conditions and on our ability to carry out our plans with respect to the development of BLU-5937, which in turn could have a material adverse effect on our business and financial condition.

Our internal computer systems, or those used by our contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems, and those of our third parties on which we rely, are vulnerable to damage from computer viruses and unauthorized access, malware, natural disasters, fire, terrorism, war and telecommunication, electrical failures, cyber-attacks or cyber-intrusions over the Internet, attachments to emails, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. While we have not experienced any such material system failure or security breach to our knowledge to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our future product candidates could be delayed.

The biopharmaceutical industry is subject to rapid technological change, which could affect the commercial viability of our products.

The biopharmaceutical industry is subject to rapid and significant technological change. Research, discoveries or inventions by others may result in medical insights or breakthroughs which render our products less competitive or even obsolete. Furthermore, there may be breakthroughs of new biopharmaceutical technologies which may become superior to ours that may result in the loss of our commercial advantage. Our future success will, in part, depend on our ability to, among others:

- develop or license new technologies that address the changing needs of the medical community; and
- respond to technological advances and changing industry standards and practices in a cost-effective and timely manner.

Developing technology entails significant technical and business risks and substantial costs. We cannot assure you that we will be able to utilize new technologies effectively or that we will be able to adapt our existing technologies to changing industry standards in a timely or cost-effective manner, or at all. If we are unable to keep up with advancements in technology, our business, financial conditions and results of operations could be materially adversely affected.

Risks Related to Future Sales or Issuances of Securities Under this Prospectus

An investor may be unable to bring actions or enforce judgments against us and certain of our directors and officers.

We are incorporated under the laws of Canada, and our principal executive offices are located in Canada. Most of our directors and officers and many of the experts named in this prospectus reside outside of the United States and all or a substantial portion of our assets and the assets of such persons are located outside the United States. Consequently, it may not be possible for an investor to effect service of process within the United States on us or those persons. Furthermore, it may not be possible for an investor to enforce judgments obtained in United States courts based upon the civil liability provisions of United States federal securities laws or other laws of the United States against those persons or us. See "*Enforcement of Judgments Against Foreign Persons or Companies*".

There is doubt as to the enforceability, in original actions in Canadian courts, of liabilities based upon United States federal securities laws and as to the enforceability in Canadian courts of judgments of United States courts obtained in actions based upon the civil liability provisions of the United States federal

securities laws. Therefore, it may not be possible for U.S. holders of common shares to enforce those actions against us, certain of our directors and officers or the experts named in this prospectus. Additionally, some of our directors and officers reside outside of Canada. Some or all of the assets of such persons may be located outside of Canada. Therefore, it may not be possible for U.S. holders of common shares to collect or to enforce judgments obtained in Canadian courts predicated upon the civil liability provisions of applicable Canadian securities laws against such persons.

The market price for our common shares may be volatile and subject to wide fluctuations in response to numerous factors, many of which are beyond our control.

The factors which may contribute to market price fluctuations of our common shares include, but are not limited to, the following:

- actual or anticipated fluctuations in our quarterly results of operations;
- recommendations by securities research analysts;
- changes in the economic performance or market valuations of companies in the industry in which we operate;
- addition or departure of our executive officers and other key personnel;
- release or expiration of transfer restrictions on outstanding common shares;
- sales or perceived sales of additional common shares;
- operating and financial performance that vary from the expectations of management, securities analysts and investors;
- regulatory changes affecting our industry generally and its business and operations;
- announcements of developments and other material events by us or our competitors;
- fluctuations to the costs of vital production materials and services;
- changes in global financial markets and global economies and general market conditions, such as interest rates and pharmaceutical product price volatility;
- significant acquisitions or business combinations, strategic partnerships, joint ventures or capital commitments by or involving us or our competitors;
- operating and share price performance of other companies that investors deem comparable to us or from a lack of market comparable companies; and
- news reports relating to trends, concerns, technological or competitive developments, regulatory changes and other related issues in our industry or target markets.

We may sell additional common shares or other securities that are convertible or exchangeable into common shares in subsequent offerings or may issue additional common shares or other securities to finance future operations or acquisitions.

We cannot predict the size or nature of future sales or issuances of securities or the effect, if any, that such future sales and issuances will have on the market price of our common shares. Sales or issuances of substantial numbers of common shares or other securities that are convertible or exchangeable into common shares, or the perception that such sales or issuances could occur, may adversely affect prevailing market prices of our common shares. With any additional sale or issuance of common shares or other securities that are convertible or exchangeable into common shares, investors will suffer dilution to their voting power and economic interest in us. Furthermore, to the extent holders of our stock options or other convertible securities convert or exercise their securities and sell the common shares they receive, the trading price of the common shares may decrease due to the additional amount of common shares available in the market.

Our management will have broad discretion with respect to the application of net proceeds received from any offering of our common shares under this prospectus.

Our management will have broad discretion in the application of the net proceeds from any offering of our common shares under this prospectus and you will not have the opportunity as part of your investment to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds of an offering, their ultimate use may vary substantially from their currently intended use. Our management may spend net proceeds received by us from a sale of our common shares in ways that do not improve our results of operations or enhance the value of our common shares or its other securities issued and outstanding from time to time. Any failure by management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business or cause the price of our securities issued and outstanding from time to time to decline.

We will incur increased costs as a result of operating as a public company in the United States and our management will be required to devote substantial time to new compliance initiatives.

As a public company, particularly after we are no longer an "emerging growth company" as defined under the JOBS Act, we will incur significant legal, accounting and other expenses that we did not incur prior to being listed in the United States. In addition, the Sarbanes-Oxley Act, and rules implemented by the SEC, and NASDAQ, impose various other requirements on public companies, and we will need to spend time and resources to ensure compliance with our reporting obligations under Canadian securities laws, as well as our obligations in the United States.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting ("ICFR"), which, after we are no longer an emerging growth company, must be accompanied by an attestation report on ICFR issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will document and evaluate our ICFR, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of our ICFR, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for ICFR. Despite our efforts, there is a risk that neither us nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our ICFR is effective as required by Section 404. This could result in a determination that there are one or more material weaknesses in our ICFR, which could cause an adverse reaction in the financial markets due to a loss of confidence in the reliability of our consolidated financial statements.

In addition, changing laws, regulations and standards relating to corporate governance and public disclosure are creating uncertainty for public companies, increasing legal and financial compliance costs and making some activities required for public company more time consuming. These laws, regulations and standards are subject to varying interpretations, in many cases due to their lack of specificity and, as a result, their application in practice may evolve over time as regulatory and governing bodies provide new guidance. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and divert management's time and attention from revenue-generating activities to compliance activities. If our efforts to comply with new laws, regulations and standards differ from the activities intended by regulatory or governing bodies, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Being a public company in the United States and complying with applicable rules and regulations will make it more expensive for us to obtain director and officer liability insurance. These factors could also make it more difficult for us to attract and retain qualified executive officers and members of our Board.

As a foreign private issuer, we are subject to different U.S. securities laws and rules than a domestic U.S. issuer, which may limit the information publicly available to our U.S. shareholders.

As a foreign private issuer under applicable U.S. federal securities laws, we are not required to comply with all of the periodic disclosure and current reporting requirements of the U.S. Exchange Act and related

rules and regulations. As a result, we do not file the same reports that a U.S. domestic issuer would file with the SEC, although we will be required to file with or furnish to the SEC the continuous disclosure documents that we are required to file in Canada under Canadian securities laws. In addition, our officers, directors and principal shareholders are exempt from the reporting and "short swing" profit recovery provisions of Section 16 of the U.S. Exchange Act. Therefore, our shareholders may not know on as timely a basis when our officers, directors and principal shareholders purchase or sell securities of the Company as the reporting periods under the corresponding Canadian insider reporting requirements are longer. In addition, as a foreign private issuer, we are exempt from the proxy rules under the U.S. Exchange Act.

The Company may lose its foreign private issuer status in the future, which could result in significant additional costs and expenses to the Company.

In order to maintain our current status as a foreign private issuer, a majority of our common shares must be either directly or indirectly owned of record by non-residents of the United States unless we also satisfy one of the additional requirements necessary to preserve this status. We may in the future lose our foreign private issuer status if a majority of the common shares are owned of record in the United States and we fail to meet the additional requirements necessary to avoid loss of foreign private issuer status. The regulatory and compliance costs to us under U.S. federal securities laws as a U.S. domestic issuer may be significantly more than the costs we incur as a Canadian foreign private issuer eligible to use MJDS. If we are not a foreign private issuer, we would not be eligible to use the MJDS or other foreign issuer forms and would be required to file periodic and current reports and registration statements on U.S. domestic issuer forms with the SEC, which are more detailed and extensive than the forms available to a foreign private issuer. In addition, we may lose the ability to rely upon exemptions from NASDAQ corporate governance requirements that are available to foreign private issuers.

EXEMPTION FROM NATIONAL INSTRUMENT 44-102

We may seek a permanent exemption from the *Autorité des marches financiers* in respect of any prospectus supplement to be filed in relation to a potential "at-the-market distribution" under this prospectus.

LEGAL MATTERS

Unless specified in the applicable prospectus supplement, certain legal matters relating to securities offered by this prospectus will be passed upon on our behalf by Davies Ward Phillips & Vineberg LLP with respect to Canadian legal matters and by Troutman Sanders LLP with respect to United States legal matters. In addition, certain legal matters in connection with an offering and sale of securities will be passed upon for any underwriters, dealers or agents by counsel to be designated at the time of such offering and sale by such underwriters, dealers or agents with respect to matters of Canadian and, if applicable, United States or other foreign law.

As of the date of this prospectus, the partners and associates of Davies Ward Phillips & Vineberg LLP, as a group, own beneficially, directly or indirectly, less than 1% of our outstanding securities of any class and less than 1% of the outstanding securities of any class of our associates or affiliates.

AUDITORS, TRANSFER AGENT AND REGISTRAR

Our auditors are KPMG LLP, Chartered Professional Accountants ("**KPMG**"), 1500 – 600, De Maisonneuve Boulevard West, Montreal, Québec, Canada, H3A 0A3.

The transfer agent and registrar for our common shares in the United States is Computershare Inc. at its principal offices located in Canton, Massachusetts. The transfer agent and registrar for our common shares in Canada is Computershare Investor Services Inc. at its offices located in Montreal, Québec.

ENFORCEMENT OF JUDGMENTS AGAINST FOREIGN PERSONS OR COMPANIES

The enforcement by investors of civil liabilities under United States federal securities laws may be affected adversely by the fact that we are incorporated under the federal laws of Canada, that most of our officers and directors are residents of Canada, that many of the experts named in this prospectus may be residents of Canada, and that most or all of our assets and the assets of said persons are located outside of the United States.

We have appointed an agent for service of process in the United States (as set forth below), but it may be difficult for holders of our common shares who reside in the United States to effect service within the United States upon those directors, officers and experts who are not residents of the United States. It may also be difficult for holders of our common shares who reside in the United States to realize in the United States upon judgments of courts of the United States predicated upon our civil liability and the civil liability of our directors, officers and experts under the U.S. federal securities laws.

Two of our directors, Franklin Berger and Chau Q. Khuong, reside outside of Canada and have each appointed BELLUS Health as agent for service of process in Canada at the following address: 275 Armand-Frappier Boulevard, Laval, Quebec H7V 4A7, Canada. Purchasers are advised that it may not be possible for investors to enforce judgments obtained in Canada against any person or company that is incorporated, continued or otherwise organized under the laws of a foreign jurisdiction or that resides outside of Canada, even if such person has appointed an agent for service of process.

We filed with the SEC, concurrently with the U.S. Registration Statement of which this prospectus is a part, an appointment of agent for service of process on Form F-X. Under the Form F-X, we appointed CT Corporation System as our agent for service of process in the United States in connection with any investigation or administrative proceeding conducted by the SEC, and any civil suit or action brought against or involving us in a United States court arising out of or related to or concerning the offering of our common shares under this prospectus.

RIGHTS OF WITHDRAWAL AND RESCISSION

Unless otherwise provided in the applicable prospectus supplement, the following is a description of a purchaser's statutory rights. Securities legislation in certain of the provinces of Canada provides purchasers with the right to withdraw from an agreement to purchase securities. This right may be exercised within two business days after receipt or deemed receipt of a prospectus and accompanying prospectus supplement relating to the securities purchased by a purchaser and any amendment thereto. In several of the provinces, the securities legislation further provides a purchaser with remedies for rescission or, in some jurisdictions, revisions of the price or damages if the prospectus and accompanying prospectus supplement relating to the securities purchased by a purchaser and any amendment thereto contains a misrepresentation or is not delivered to the purchaser, provided that the remedies for rescission, revisions of the price or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province for the particulars of these rights or consult with a legal advisor.

CERTIFICATE OF THE COMPANY

Date: January 17, 2020

This short form base shelf prospectus, together with the documents incorporated in this prospectus by reference, constitutes full, true and plain disclosure of all material facts relating to the securities offered by this prospectus as required by the securities legislation of each of the provinces of Canada.

(Signed) ROBERTO BELLINI
President and Chief Executive Officer

(Signed) FRANÇOIS DESJARDINS
Vice President, Finance

On behalf of the Board of Directors

(Signed) FRANCESCO BELLINI
Director

(Signed) PIERRE LAROCHELLE
Director