
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934
Date of Report (Date of earliest event reported): **November 27, 2018**

Arsanis, Inc.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-38295
(Commission
File Number)

27-3181608
(IRS Employer
Identification No.)

890 Winter Street, Suite 230
Waltham, Massachusetts
(Address of principal executive offices)

02451
(Zip Code)

Registrant's telephone number, including area code: **(781) 819-5704**

Not applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 8.01. Other Events.

On November 27, 2018, Arsanis, Inc. (“Arsanis”) and X4 Pharmaceuticals, Inc. (“X4”) held a joint investor conference call to discuss their previously announced entry into an agreement and plan of merger. A transcript of the conference call is filed as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) *Exhibits*

| <u>Exhibit No.</u> | <u>Description</u> |
|--------------------|--|
| 99.1 | <u>Transcript of joint investor conference call held by Arsanis, Inc. and X4 Pharmaceuticals, Inc. on November 27, 2018.</u> |

PARTICIPANTS IN THE SOLICITATION

Arsanis, X4 and their respective directors and executive officers may be deemed to be participants in the solicitation of proxies from the holders of Arsanis common stock in connection with the proposed transaction. Information about Arsanis’ directors and executive officers is set forth in Arsanis’ Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the Securities and Exchange Commission (the “SEC”) on March 9, 2018, and the proxy statement for Arsanis’ 2018 annual meeting of stockholders, which was filed with the SEC on April 23, 2018. Other information regarding the interests of such individuals, as well as information regarding X4’s directors and executive officers and other persons who may be deemed participants in the proposed transaction, will be set forth in the proxy statement/prospectus/information statement, which will be included in Arsanis’ registration statement when it is filed with the SEC. Investors and security holders may obtain free copies of these documents as described in the paragraph below.

IMPORTANT INFORMATION ABOUT THE TRANSACTION WILL BE FILED WITH THE SEC

In connection with the proposed merger, Arsanis will file with the SEC a Registration Statement on Form S-4 that will include a proxy statement of Arsanis, a prospectus of Arsanis and an information statement of X4 and certain of its affiliates (the “X4 Parties”), and the parties may file with the SEC other relevant documents concerning the proposed transaction. Arsanis will mail the definitive proxy statement/prospectus/information statement to the Arsanis stockholders and the X4 Parties equity holders. ARSANIS STOCKHOLDERS AND X4 PARTIES EQUITY HOLDERS ARE URGED TO READ THE REGISTRATION STATEMENT AND PROXY STATEMENT/PROSPECTUS/INFORMATION STATEMENT REGARDING THE PROPOSED TRANSACTION WHEN IT BECOMES AVAILABLE AND ANY OTHER RELEVANT DOCUMENTS FILED WITH THE SEC, AS WELL AS ANY AMENDMENTS OR SUPPLEMENTS TO THOSE DOCUMENTS, BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION. Investors and security holders may obtain a free copy of the proxy statement/prospectus/information statement (when available) and other filings containing information about Arsanis at the SEC’s website at www.sec.gov. The proxy statement/prospectus/information statement (when available) and the other filings may also be obtained free of charge by contacting: Arsanis, Inc., 890 Winter Street, Suite 230, Waltham, Massachusetts 02451, Attention: Investor Relations.

Additional information regarding the interests of those participants and other persons who may be deemed participants in the transaction may be obtained by reading the proxy statement/prospectus/information statement regarding the proposed transaction when it becomes available. Free copies of this document may be obtained as described in the preceding paragraphs.

NO OFFERS OR SOLICITATIONS

This communication shall not constitute an offer to sell or the solicitation of an offer to sell or the solicitation of an offer to buy any securities, nor shall there be any sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such jurisdiction. No offer of securities shall be made except by means of a prospectus meeting the requirements of Section 10 of the Securities Act of 1933, as amended (the "Securities Act").

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Certain statements in this Current Report on Form 8-K and the exhibit attached hereto regarding both the proposed merger and other contemplated transactions (including statements relating to satisfaction of the conditions to and consummation of the proposed merger; the expected ownership of the combined company; the alternatives to the proposed merger; the expected benefits of the merger; the management and organization of the combined company; the initiation, cost, timing, progress and results of X4's development activities, nonclinical studies and clinical trials; the potential benefits that may be derived from any product candidates; X4's strategy to advance strategic collaborations; and the strategies, goals, prospects, plans, expectations, forecasts or objectives of Arsanis, X4 or the combined company) constitute "forward-looking statements" within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," "would," and variations of such words or similar expressions. Arsanis intends for these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act and is making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect Arsanis' current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to Arsanis and on assumptions it has made. Although Arsanis believes that its plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, Arsanis can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of important risks and factors that are beyond Arsanis' control.

Risks and uncertainties for Arsanis, X4 and the combined company include, but are not limited to, the: inability to complete the proposed merger and other contemplated transactions; liquidity and trading market for shares prior to and following the consummation of the proposed merger; costs and potential litigation associated with the proposed merger; failure or delay in obtaining required approvals by the SEC or any other governmental or quasi-governmental entity necessary to consummate the proposed merger, including Arsanis' ability to file an effective proxy statement/prospectus/information statement in connection with the proposed merger and other contemplated transactions, which may also result in unexpected additional transaction expenses and operating cash expenditures on the parties; failure to obtain the necessary stockholder approvals or to satisfy other conditions to the closing of the proposed merger and the other contemplated transactions; a superior proposal being submitted to either party; failure to issue Arsanis' or the combined company's common stock in other contemplated transactions exempt from registration or qualification requirements under applicable state securities laws; risks related

to the costs, timing and regulatory review of the combined company's nonclinical studies and clinical trials; uncertainties in obtaining successful clinical results for product candidates such as X4's X4P-001 and unexpected costs that may result therefrom; inability or the delay in obtaining required regulatory approvals for product candidates such as X4P-001, which may result in unexpected cost expenditures; failure to realize any value of certain product candidates developed and being developed, in light of inherent risks and difficulties involved in successfully bringing product candidates to market; inability to develop new product candidates; inability to commercialize and launch any product candidate that receives regulatory approval, including X4P-001; the combined company's anticipated capital expenditures, its estimates regarding its capital requirements and its need for future capital; uncertainties of cash flows and inability to meet working capital needs; cost reductions that may not result in anticipated level of cost savings or cost reductions prior to or after the consummation of the proposed merger; the approval by the U.S. Food and Drug Administration and European Medicines Agency and any other similar foreign regulatory authorities of other competing or superior products brought to market; risks resulting from unforeseen side effects; risk that the market for the combined company's products may not be as large as expected; inability to obtain, maintain and enforce patents and other intellectual property rights or the unexpected costs associated with such enforcement or litigation; inability to obtain and maintain commercial manufacturing arrangements with third party manufacturers or establish commercial scale manufacturing capabilities; inability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and the inability to attract collaborators with development, regulatory and commercialization expertise; inability to successfully commercialize any approved product candidates, including their rate and degree of market acceptance; unexpected cost increases and pricing pressures; the possibility of economic recession and its negative impact on customers, vendors or suppliers; and risks associated with the possible failure to realize certain benefits of the proposed merger, including future financial, tax, accounting treatment, and operating results. Many of these factors that will determine actual results are beyond Arsanis', X4's, or the combined company's ability to control or predict.

Other risks and uncertainties are more fully described in Arsanis' Annual Report on Form 10-K for the year ended December 31, 2017, filed with the SEC, and in other filings that Arsanis makes and will make with the SEC in connection with the proposed transactions, including the proxy statement/prospectus/information statement described herein under "Important Additional Information About the Transaction Will be Filed with the SEC." Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. The statements made in this Current Report on Form 8-K and the exhibit attached hereto speak only as of the date stated herein, and subsequent events and developments may cause Arsanis' expectations and beliefs to change. While Arsanis may elect to update these forward-looking statements publicly at some point in the future, it specifically disclaims any obligation to do so, whether as a result of new information, future events or otherwise, except as required by law. These forward-looking statements should not be relied upon as representing Arsanis' views as of any date after the date stated herein.

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ARSANIS, INC.

Date: November 27, 2018

By: /s/ Michael P. Gray

Michael P. Gray
President and Chief Executive Officer and
Chief Financial Officer



**Arsanis, Inc. and X4
Pharmaceuticals, Inc.
Conference Call**

Tuesday, November 27th, 2018

Operator: Good day, ladies and gentlemen, and welcome to the Arsanis and X4 Pharmaceuticals Conference Call. At this time, all participants are in a listen-only mode. Later, we will conduct a question-and-answer session and instructions will be given at that time. If anyone should require assistance during the conference, please press star then zero to reach an operator. As a reminder, this call is being recorded.

I would now like to turn the call over to Mike Gray. You may begin.

Transaction Overview

Michael Gray

President and CEO, Arsanis, Inc.

Opening remarks

Okay. Thank you, Michelle. Good morning and thanks to everyone for joining us today to discuss the proposed merger of Arsanis and X4 Pharmaceuticals. Joining me on the call this morning are X4's President and CEO, Dr. Paula Ragan, and X4's CFO, Adam Mostafa.

Please note, in addition to the press release issued this morning to announce the merger, we have included presentation slides in the webcast of today's call and posted a PDF of the slides on the website of both companies. If you have not already done so, I encourage you to open the webcast and our presentation PDF to follow along with our prepared remarks this morning.

Safe harbor statement

Turning to Slide Two. Slide Two provides an overview of our forward-looking statements. I'd like to remind everyone that our call today will include remarks about future expectations, plans and prospects for Arsanis and X4, which constitute forward-looking statements for the purpose of the safe harbor provisions under applicable Federal Securities laws.

These forward-looking statements include, without limitation, statements regarding the proposed merger and other contemplated transactions, including statements relating to the satisfaction of the conditions to and consummation of the proposed merger, the expected ownership of the combined company, the alternative to the proposed merger, the expected benefits of the merger, the management and organization of the combined company, the initiation cost, timing, progress, and results of X4's development activities, non-clinical studies and clinical trials, the potential benefits that may be derived from any product candidates, X4's strategy to advance strategic collaborations and the strategies, prospects, plans, expectations, forecasts or objectives of Arsanis X4, the combined company. These forward-looking statements involve significant risks and uncertainties that could cause actual results to differ materially from those expected, including those listed on Slide Two of this presentation.

Participants and Additional Information

Turning to Slide Three. And as outlined on this slide, please be advised to read, when available, Arsanis filings with the SEC, including a registration statement that will contain a proxy statement and a prospectus of Arsanis and an information statement of X4 Pharmaceuticals because these documents will contain important information about the

transaction and the participants' interest in such transactions. These documents can be obtained without charge by contacting Arsanis, once these filings are complete, at the address provided on Slide Three or at the SEC's internet website, which is www.sec.gov.

Before I begin prepared remarks on Slide Four, I just want to say a quick personal remark. As you may have noted from this morning's press release, René has stepped down, as President and CEO, to pursue other opportunities and I'll be taking over this role, effective today. René has been a great leader for Arsanis and on behalf of the entire Arsanis team, I want to thank René for this leadership as CEO over the last two and a half years.

The Arsanis board and I are pleased that René has agreed to continue to serve on our board and is also expected to be one of the two Arsanis board Representatives for the combined company. So, she'll remain an important part of Arsanis and will continue to represent Arsanis and its stockholders as a member of the X4 board, following transaction closing.

Transaction overview

So now, turning to Slide Four contents, I'll begin with an overview of the transaction and then I'll turn it over to Dr. Ragan for an introduction of the X4 business.

For anyone who's not familiar with Arsanis, we had been conducting a Phase 2 clinical trial of our lead asset, ASN100, for the prevention of staph pneumonia in high-risk mechanically-ventilated patients. We announced the discontinuation of this Phase 2 clinical trial based on results from a planned interim analysis of unblinded trial data.

Shortly thereafter, we began to consider strategic options with our board of directors to maximize shareholder value. We engaged Leerink as part of this process and worked closely with Leerink and our board to conduct a formal process that culminated in an extensive and thorough review of several proposals.

As a result of this process, we concluded that this merger with X4 represents the best path forward for Arsanis stockholders by providing a significant equity position on what we believe is a promising clinical stage biopharmaceutical company developing novel therapies for multiple rare diseases of the immune system as well as rare cancers.

Combined company will operate as X4 Pharmaceuticals

The combined company will operate as X4 Pharmaceuticals and expects to initiate a Phase 3 clinical trial for its lead product candidate, X4P-001 in WHIM syndrome in the first half of 2019. X4's infrastructure will be strengthened by Arsanis' clinical development and regulatory employees in our Vienna R&D facility.

Paula will serve as President and CEO of the combined company and its board of directors will include five members from X4, including the chairperson, and two members from Arsanis. As I mentioned, René is expected to be one of those directors from Arsanis as well as Dave McGirr, who is our Audit Committee Chair as well.

Transaction expected to close by Q1 2019

The merger has been approved by the boards of both companies and we have secured voting agreements supporting the transaction from stockholders representing approximately 48% of Arsanis' common stock. We anticipate the transaction will close in the first quarter of 2019, subject to the approval of the stockholders of both companies and the satisfaction or waiver of other customary conditions.

Expected ownership split at the time of transaction closing

At closing, we expect X4 stockholders will own about 70% of the combined company and Arsanis stockholders will own the balance of 30%. The exchange ratios for X4 common stock and X4 preferred stock are set forth in the merger agreement and assume a pre-transaction valuation for X4 of \$115 million and \$50 million Arsanis' business. However, in the case for Arsanis, the pre-transaction valuation is subject to downward or upward adjustment to the extent Arsanis' net cash as of the business day prior to closing is above or below \$20 million.

Arsanis' evaluation of \$50 million is based on projected available cash at closing, the value of certain aspects of our organization, including the clinical and regulatory teams in the Vienna research development site, the potential of certain of Arsanis' programs, including potential payments from partner programs as well as the value of our NASDAQ listing. X4's assumed value of \$115 million is based on the value of its pipeline, the valuation of its last private round, and also X4's achievement of various milestones since completing that round. In addition, reference was made to other comparable publicly-traded rare disease companies.

That concludes my prepared remarks on the transaction. And I'd like to now turn the call over to Dr. Ragan for an overview of X4.

Overview of X4 Pharmaceuticals

Dr. Paula Ragan

President and CEO, X4 Pharmaceuticals, Inc.

Opening remarks

Thank you, Mike, and good morning, everyone. I'm very pleased to be here today to announce this transformative event for both companies and to share the X4 story. We believe this merger represents a unique opportunity to provide capital to support our Phase 3 clinical trial of X4P-001 in WHIM syndrome, advance our other pipeline program, expand our future financing options, and enhance our team and infrastructure, particularly in Europe, which supports our corporate objective of conducting a global Phase 3 clinical trial in our lead program, X4P-001, and its subsequent global launch in commercialization.

Company overview

Moving on to Slide Five. Here, we show a high-level overview of X4. Our mission is to develop treatments designed to have a clear and profound impact for patients suffering with rare diseases, including WHIM syndrome, and patients with rare cancers.

We seek to achieve this mission through the development of novel therapeutics designed to improve immune cell trafficking. The company was founded in 2014 in Cambridge, Massachusetts, based on assets developed at Genzyme. We've raised \$75 million to date and our largest investor is Comorant Asset Management.

Building a global rare disease franchise

Turning to Slide Six. We believe X4 is positioned to rapidly create a global rare disease franchise built on the following key pillars:

- First, we have the opportunity for a rapid path to commercialization for our therapies,

given our rare disease focus.

- Second, we have multiple programs with anticipated clinical readouts of plans for the next several years culminating in the Phase 3 topline results for our lead program in 2021.
- Third, we have a pipeline of early stage and preclinical opportunities across the rare disease landscape that provide expansion opportunities.
- And fourth, in total, we estimate there's more than \$1 billion of identified market opportunities through our existing pipeline.
- And finally, we have assembled a strong leadership team with a rare disease expertise, including many people that were formerly members of the Genzyme team.

Proven leadership team with rare disease expertise

So, on to Slide Seven. We've provided an overview of our team which includes this deep rare disease experience across the executive team, board and founders.

Prior to founding X4, I consulted as the Chief Business Officer of Lysosomal Therapeutics. And before that, I held various leadership roles in corporate development and operations at Genzyme Sanofi, including leading Genzyme's rare disease business, strategic partnering efforts and the supply chain planning for Genzyme's flagship commercial products. Importantly, during my time at Genzyme, the company developed and launched Mozobil, a treatment delivered solely by injection and the only approved CXCR4 antagonist.

Our management team and board of directors bring a breadth of experiences in the development and commercialization of rare disease products launched by companies such as Genzyme, Alexion, Sarepta and Biogen: specifically, Mike Wyzga, X4's Chairman of the Board, was a prior Chief Financial Officer of Genzyme for over a decade. Gary Bridger, an X4 Director, was the prior Chief Scientific Officer at AnorMED, a company which was acquired by Genzyme. Gary led the Research and Development efforts, which ultimately led to the many global approvals of Mozobil.

Finally, I'd like to recognize the role that Henri Termeer played in X4. Henri, who pioneered treatments for rare diseases and led Genzyme as a CEO for over 25 years, was one of the co-founders of X4, helping the company begin its journey. His inspiration and mission live on in me and in X4 Pharmaceuticals.

Pipeline

X4P-001

On Slide Eight, we provided an overview here of our pipeline. Our lead product candidate is X4P-001, an oral small molecule that we are initially investigating across rare immunodeficiency indications and in oncology. We expect to initiate the Phase 3 clinical trial of X4P-001 for WHIM syndrome in the first half of 2019 with topline results targeted for 2021.

Also, in the first half of next year, we plan to initiate Phase 1/2 study for X4P-001 in severe congenital neutropenia (SCN) and Waldenstrom's macroglobulinemia. We've also completed Phase 1B clinical trials of X4P-001 for the treatment of melanoma in renal cell carcinoma (RCC) in combination with checkpoint inhibitors. These studies demonstrated acceptable safety profiles, proof of mechanism and favorable clinical activity. Additionally, we have a

second ongoing Phase 2A study in RCC in combination with axitinib, which we expect to read out in mid-2019.

In the second half of 2019, we aim to file INDs for our next two product candidates, X4P-003 in primary immunodeficiencies and X4P-002 in brain cancers, namely glioblastoma.

Strategy focused on building a strong diversified commercial rare disease business

On to Slide Nine. So as a result, we have a strong cadence of anticipated value-building milestones over the both near and longer term, as outlined here. These expected milestones are staggered across our pipeline over the next few years, potentially expanding our opportunities in rare disease and cancers as we build towards the Phase 3 topline results of our lead program. Importantly, we also have the potential to advance some partnerships with our oncology programs as well.

CXCR4/CXCL12 and immune system responses

On to Slide Ten. So with that high-level overview of the company, our opportunities and development plans, now let's drive a bit more detail into the underlying biology of our key program.

The diagram on this slide shows the relationship between the chemokine CXC Receptor Type 4 (CXCR4) and a full ligand, CXCL12. This receptor-ligand pair is well-known to play in an essential role in normal trafficking, maturation and surveillance of immune cells. In the setting of various diseases, we believe the overstimulation of the receptor-ligand pair results in impairment of normal signaling functions and results in decreased trafficking of these key immune cells. This can ultimately lead to increased infections and the promotion of cancer cell survival.

Our lead program, X4P-001, is an orally delivered small molecule that blocks overstimulation of the receptor-ligand pathway and aims to restore functional immune surveillance. We believe X4P-001 is the only oral CXCR4 foreign antagonist in clinical development. We believe it is also the only oral allosteric inhibitor of the receptor, which can potentially be the best approach for inhibiting overstimulation. And finally, it's administered as a single once-daily dose.

Of the many diseases that are connected to this key area of human biology, X4 has initially focused on a number of rare diseases in which genetic mutations in the CXCR4 receptor gene and its related pathways interfere with the normal trafficking of key immune cells.

Overview: XP4-001 for WHIM

WHIM: Warts, Hypogammaglobulinemia, Infections and Myelokathexis

So, on to Slide 11. One of these genetic rare diseases, known as WHIM syndrome, is driven by, again, a function mutation in the CXCR4 gene. Patients with genetic defects in CXCR4 have profoundly low white cell blood counts. They are severely neutropenic and lymphopenic, leaving them susceptible to infections and increased risk of certain cancers throughout their lives.

WHIM is an abbreviation for the characteristic clinical symptoms of the syndrome: Warts, Hypogammaglobulinemia, Infections and Myelokathexis. On the right-hand of the slide, you can see a hand covered in warts from a WHIM patient whose immune system is not able to

clear the human papillomavirus (HPV), a virus associated with certain cancers. And below that image is a picture of a WHIM patient that developed HPV-associated oral cancer. A portion of his jaw had to be removed as part of his cancer treatment.

Current therapy

Unfortunately, WHIM syndrome is also associated with significant morbidity beginning in early childhood and continuing throughout life. Current therapy is limited to treatment of acute infections with antibiotics, intravenous immunoglobulins, or G-CSFs is also sometimes used for the prevention of infection, although neither treatment has been clinically shown to benefit WHIM patients.

Our lead product candidate, X4P-001, has the potential to provide disease-modifying treatment for WHIM patients, given its mechanism. We believe this mechanism directly addresses the root cause of WHIM syndrome by inhibiting the “gain-of-function” effects of the CXCR4 gene mutation.

Proof of concept in WHIM demonstrated with Mozobil

Proof of concept for this approach in WHIM patients has been previously demonstrated with twice-daily injections of Mozobil, a CXCR4 antagonist that members of our team at Genzyme helped develop. In previous studies, these twice-daily injections of Mozobil increased neutrophil and lymphocyte counts and reduced infection rates in wart lesions in a small pilot study in WHIM patients. And Mozobil is not approved for the treatment of WHIM syndrome.

Orphan Drug Designation from FDA for X4P-001, partnerships to raise awareness and build patient registries

In October 2018, we received Orphan Drug Designation from the FDA for X4P-001 for the treatment of WHIM syndrome. And earlier this year, we published promising results from our Phase 2 study in WHIM and we expect to enter a Phase 3 study in the first half of next year.

In parallel, we are partnering with several foundations and institutions to increase the awareness and diagnosis of WHIM and build our patient registries. We believe the worldwide market opportunity for WHIM syndrome to be approximately \$500 million.

More than 1,000 WHIM patients estimated in US as per X4 primary research

212 MDs reported 1,772 WHIM patients and 24 MDs reported 62 genetically-confirmed WHIM patients

On to Slide 12. This slide provides a summary of a 2017 survey of US physicians across five specialties that were most commonly involved in the care of WHIM patients. From this survey, 212 physicians reported an aggregate of 1,772 patients with either genetically-confirmed WHIM syndrome or that were highly suspected of having WHIM. A subset analysis from this survey showed 24 physicians cared for 62 genetically-confirmed patients or an average of 2.6 patients per specialist. And based on this, we estimate today that there are at least 1,000 genetically-confirmed WHIM patients in the US.

With additional education and awareness, we expect the worldwide diagnosis of WHIM syndrome to grow significantly over the coming years and weigh similar to other products that have successfully treated other rare genetic diseases.

WHIM: genetic mutations in CXCR4 create abnormal trafficking of white blood cells

Now, I'll spend more time on the connection between the genetic mutation and the resulting clinical profile of WHIM patients. Slide 13 illustrates the immune function of a normal subject, a WHIM patient, and a WHIM patient treated with X4P-001.

In the left panel, you can see that within the bone marrow, a normally functioning CXCR4 receptor controls the release of white blood cells, including neutrophils and lymphocytes, into the bloodstream, thereby enabling normal immune surveillance functions throughout the body.

In patients with WHIM syndrome, represented in that middle panel, mutations to the CXCR4 gene results in deleting a portion of the receptor as shown by that small red X. This causes the abnormal signaling and leads to the retention of all forms of white blood cells, including neutrophils and lymphocytes, in the bone marrow and, therefore, inadequate immune surveillance and function.

In the right panel, we believe treatment with X4P-001 normalizes the signaling for the mutant CXCR4 receptor to promote the release of white blood cells, thereby restoring a more healthy and functional immune system.

Proof of concept in WHIM previously demonstrated with Mozobil

As I had mentioned, proof of concept for X4P-001 in WHIM syndrome was established by Mozobil previously. This was the use of twice-daily injectable form of the CXCR4 antagonist, which was discovered, developed and commercialized by a team at Genzyme, including certain X4 founders and board members. This study provided compelling evidence for the disease-modifying potential of X4P-001 and was the basis for our clinical approach.

Phase 2 study design: neutrophil counts biomarker

Phase 2 goal: daily neutrophil and lymphocyte counts exceed target thresholds

Slide 14 outlines our Phase 2 study design for X4P-001 in WHIM. We conducted an open-label dose-titration study to evaluate safety end-measures in pharmacokinetics and pharmacodynamics and assess biomarkers of 24-hour blood counts of neutrophils and lymphocytes – important sub-types of white blood cells. The goal of this clinical trial was to determine if cell counts could be increased beyond predefined thresholds, which we believe to be clinically meaningful.

In the prior Mozobil proof of concept study, WHIM patients who showed on-treatment increases in blood counts to these thresholds also showed clinical improvement in reduction in infection rates and decreases in wart lesions.

Phase 2: achieved threshold targets in most patients

DRC recommended 400 mg QD for Phase 3 trial

Slide 15. I'll now share the preliminary results from our Phase 2 study in WHIM patients. As you can see in the graph, on the top of the Slide 15, we observed a dose-dependent increase in neutrophils, with about half of the patients achieving the target biomarker level at a 300-milligram dose. For those not achieving target, daily doses were increased to 400 milligrams and nearly all achieved target neutrophil level. Lymphocyte counts achieved target level in almost all patients as well.

X4P-001 was observed to have a safety profile – based positive safety profile and to be well-

tolerated at all doses. So based on the safety profile along with the results of dose-dependent neutrophil and lymphocyte activity, the Data Review Committee recommended that a 400-milligram daily dose of X4P-001 be utilized in the Phase 3 study.

We presented interim results from the Phase 2 trial of X4P-001 in WHIM at the EHA Meeting in June of this year. And we will present additional data from the Phase 2 trial in a poster presentation at the ASH Meeting in San Diego this Saturday.

Dramatic reduction in wart burden consistent with disease-modifying MOA

On to Slide 16. In addition to the positive safety data and the achievement of our threshold count, we saw preliminary evidence of clinical activity in the form of anecdotal reductions in infection rates and reduction in wart lesions.

On Slide 16, we present images provided by our investigator, showing the wart lesions of Patient 006. Prior to entering the study, Patient 006 was reported to have a long history of severe wart lesions that were refractory to all available treatments. In these images, one can see the dramatic reduction of wart burden observed in Phase 2 study at 26 weeks of treatment with X4P-001. This patient had no treatment of any kind, during the study, for warts. And the reductions in her wart lesions were reported by the investigator as probable drug effects of X4P-001. And this patient continues to be treated today in the expansion arm of the study, and updates will be presented at ASH.

Phase 3 trial of X4P-001 in WHIM – expected initiation in H1 2019

On to Slide 17. So, based on these positive Phase 2 results, we plan to initiate a Phase 3 study of X4P-001 in the first half of 2019. We have outlined the anticipated protocol for the study on Slide 17. We expect it will be a one-to-one randomization of nine WHIM patients to treatment and nine WHIM patients to placebo.

We plan to follow these patients for one year to assess changes in white blood cell counts as well as clinical metrics. And upon completion of the trial, all patients will be allowed to roll over into an open-label phase of the study and be provided treatment with X4P-001. Based on this, we expect topline data from the randomized portion of the study in 2021.

X4P-001: additional opportunities

So, in addition to WHIM syndrome, we have identified two other indications that we believe can benefit from the treatment of X4P-001.

Severe congenital neutropenia

The first is severe congenital neutropenia (SCN), which is a rare primary immunodeficiency with an estimated prevalence in the US and EU today of approximately 2,000 to 3,000 patients. Similar to WHIM, SCN is characterized by abnormally low levels of neutrophils, chronic severe infections and, in some, increased blood cancers. Certain subtypes of SCN have mechanisms that overlap with the signaling of the CXCR4 pathway. Therefore, patients may benefit from the treatment with X4P-001.

Waldenstrom's macroglobulinemia

The second indication is Waldenstrom's macroglobulinemia, which is a rare form of non-Hodgkin's lymphoma with an estimated prevalence of more than 13,000 patients in the US and Europe combined. We estimate that greater than 90% of Waldenstrom's patients have

mutations in the MYD88 gene and a good portion can be effectively treated with ibrutinib.

However, somewhere between 30% to 40% of all Waldenstrom's patients are poor responders to ibrutinib. These poorly responsive patients have then shown to add a gain-of-function, WHIM-like mutation in the CXCR4 gene in the cancer cells that's defined as a very rare form of lymphoma. So, we're in the planning stages of our Phase 1/2 study in Waldenstrom's and expect to initiate the trial in the first half of 2019.

Market opportunities

We estimate that Waldenstrom's represents a \$300 million market opportunity and SCN represents a \$150 million market opportunity, globally. And outside of X4P-001, we believe X4P-003 can expand and extend our efforts in additional immunodeficiency indications; and X4P-002, which is a drug designed to penetrate the blood-brain barrier, can provide additional upside in rare forms of brain cancers also shown to be strongly linked to CXCR4.

IO strategy: leveraging biological expertise via partnering

Three ongoing trials demonstrating proof of concept

So on to Slide 19. Turning back to X4P-001, we are also investigating the drug candidate in several clinical studies in solid tumors. We believe it can help restore healthy immune cell trafficking within the tumor microenvironment and has the potential to enhance the anti-tumor activity of approved oncology agents such as checkpoint inhibitors and targeted therapies.

Additional clinical collaboration expected near-term for checkpoint inhibitors

We have ongoing efforts to demonstrate this mechanism and proof of concept in oncology as listed here on Slide 19. Recently, we've reported out the results of two of our Phase 1B studies, demonstrating proof of mechanism and clinical activity. The most advanced of the three trials is in a 65-patient Phase 2A study in advanced RCC in a clinical collaboration with Pfizer. Our strategy is to secure additional clinical collaborations and to ultimately establish a strategic partnership that could provide non-dilutive financing.

Key benefits of the merger

On to Slide 20. So in closing, we believe X4 has a significant opportunity to build a robust global rare disease franchise targeting more than \$1 billion market potential, spearheaded by X4P-001 in WHIM syndrome. We anticipate that our merger with Arsanis leverages our combined strengths, provides near-term capital to support our Phase 2 trial, and further strengthens our team in infrastructure as X4 Pharmaceutical seeks to continue to grow and pursue our strategy to become a global commercial organization.

And with that, I'll now turn the call over to the operator for the Q&A session. Thank you.

Q&A

Operator: Ladies and gentlemen, if you'd like to ask a question, please press star then one. If your question has been answered and you'd like to remove yourself from the queue, you may press the pound key. Once again, to ask a question, please press star then one.

Our first question comes from Stephen Willey of Stifel. Your line is open.

Stephen Willey (Stifel): Yeah, good morning. Thanks for taking the questions, and congrats on the transaction. Paula, I was maybe just wondering if you can just speak a little bit to some of the use of supportive care that either may or may not be allowed within the planned Phase 3 trial? And I guess I'm thinking of things like IgG.

Dr. Paula Ragan: Yeah. Thanks for the question, Steve. So, during the Phase 2 portion of the trial, we typically will not allow patients on IVIg. But for a small number of patients – so, we are going to balance out the arms. And about one-third of the patients will be allowed to be maintained on IVIg and about two-thirds will not, just to balance out and be consistent with standard of care, which is slightly different in the US and Europe.

G-CSF will not be allowed to be used during the course of the trial, but for rescue therapy. And we will be ensuring that patients kind of return to baseline, if they're coming back for any of their plans, on measurements or assessments during the trial.

Stephen Willey: Okay. That's helpful, thank you. And then, I know that there are other CXCR4 antagonists that are in development – I think mostly positioned towards oncology. Could you maybe just talk a little bit about how 001 is kind of competitively different from some of these other antagonists that are in development?

Dr. Paula Ragan: Yeah. Thanks again for the question. So, there's two main points that differentiate us:

- Number one, we are the only oral treatments. All other CXCR4 antagonists are large-molecule and are either subcutaneous or delivered through intravenous infusion.
- The second important point is the science of mechanism. All the other available or clinically tested CXCR4 antagonists actually compete with the ligand-binding pockets and our drug is an allosteric inhibitor.

We know there's actually some very robust science that the team has completed and we believe this actually more robustly shut down receptor signaling irrespective of the amount of ligands that is present in any of these microenvironments. So again, we believe that we have sort of a better mousetrap for this approach.

Stephen Willey: Great. And then, just lastly for me, I know that 002 is distinct in the sense that it's CNS-penetrant. But can you just remind us how 003 is distinct from 001 and how this is, I guess, a better candidate for some of the primary immunodeficiency diseases you intend to pursue this in? Thanks.

Dr. Paula Ragan: Yeah. Yeah, So, 003 – the target product profile for 003 is to improve some of the PK/PD profiles, which could potentially reduce dosing long-term. The other element that we'd like to engineer out is that 001 has a food effect. So, patients today will be taking it on an empty stomach once a day.

While it doesn't seem to have a major impact in terms of compliance, obviously; for lifelong treatment, we would very much like to engineer that out. So 003, again, is kind of a next-generation molecule that could certainly expand compliance and the broader footprint for any patients who can't tolerate the required food restrictions.

Stephen Willey: Very helpful. Thanks for taking the questions.

Dr. Paula Ragan: Thank you.

Operator: Our next question comes from Joel Beatty of Citi. Your line is open.

Joel Beatty (Citi): Hi. Congrats on the transaction, and thanks for taking the questions. The first one is on WHIM. Would all patients – regardless of the mutation, location and types – be amenable to treatment with your lead agent or does it depend on the specific mutation?

Dr. Paula Ragan: Oh, great. Thanks for the question. So, to our knowledge, all of the mutations of the CXCR4 gene actually occur on the intracellular arm of the receptor. Our drug acts extracellularly on the transmembrane regions 1 and 3. So, we fully expect the drug to be broadly applicable to all of the known forms of CXCR4 gene mutation.

Joel Beatty: Great. And then also, could you discuss how your drug compares to Mozobil for WHIM, you know, in terms of the efficacy signings and tolerability and so forth?

Dr. Paula Ragan: Yeah. So, it's a great question. So, just a bit of history, Mozobil was actually discovered and developed by the same founder and board member, Gary Bridger. So, we are very, very familiar with Mozobil head-to-head against our molecule almost in any assay you could consider.

So, I would say the good news is at the receptor level, the drugs acted very similarly once the drug actually hits the receptor. They're actually overlapping quite similarly, in terms of the allosteric binding sites. So, the unique profiles is that Mozobil is only available through IV infusions or sub-cu infusions. It's rapidly cleared through the kidneys, so it has a four-hour half-life.

Our drug is orally available. It has 22-hour half-life. Therefore, it's much more amenable to the once-daily treatment that we think is most sustainable for rare disease patients to take throughout their lives.

Joel Beatty: Great. And then, the last question is just a follow-up to the food interaction you mentioned. Could you describe how long a patient needs to fast with your drug?

Dr. Paula Ragan: I think the clinical trial right now is restricting one hour before and two hours after the dose. So typically, patients take it first thing in the morning.

Joel Beatty: Got it. Thanks for the questions.

Dr. Paula Ragan: Thank you.

Joel Beatty: Thank you.

Operator: Once again, if you'd like to ask a question, please press star then one. There are no further questions. I'd like to tum the call back over to Paula Ragan for any closing remarks.

Dr. Paula Ragan: So, I would just like to say thank you to everyone who attended today's call. And thanks to the Arsanis team as well; we're very excited about the future ahead of us. And thanks again.

Operator: Ladies and gentlemen, thank you for participating in today's conference. This does conclude the program, and you may all disconnect. Everyone have a great day.

[END OF TRANSCRIPT]