Alexion Highlights Promising Pipeline & Distinguished Rare Disease Capabilities at Virtual Investor Day

October 6, 2020

- Robust pipeline of 20+ development programs across 7 rare disease franchises with future plans to continue growing pipeline with >5 novel INDs by 2025 -

- Increasing depth and breadth of growth opportunities expected to generate 2025 global revenues of $9-10 billion & >10% revenue CAGR through 2025 and beyond -

- Expect to raise 2020 full-year revenue guidance by >$200 million -

- Robust share repurchase commitment expected to return ~$3 billion cash to shareholders through 2023 -

BOSTON--(BUSINESS WIRE)--Oct. 6, 2020-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced continued progression of the company's LEAD-EXPAND-DIVERSIFY value-creation strategy and will highlight strategic advancements at today's Virtual Investor Day. The day will be led by Alexion's Chief Executive Officer Ludwig Hantson, Ph.D., who will be joined by Board Chairman David Brennan and other members of the leadership team, in a series of presentation and Q&A sessions to provide further insight into the company's robust pipeline, future growth potential and continued progress advancing its mission of improving the lives of people with rare diseases and devastating conditions.

The day will highlight select strategic programs to illustrate the significant promise of Alexion's portfolio and its value-creating potential, which includes:

- Robust pipeline of more than 20 development programs across seven rare disease franchises, with expected continued growth from more than five novel investigational new drug applications (INDs) by 2025
- Anticipated 2025 global revenue target of $9 to $10 billion, and at least 10 percent revenue compound annual growth rate (CAGR) through 2025 and beyond
- Plan to raise 2020 full-year revenue guidance by more than $200 million when reporting third quarter results
- Expect to return at least $3 billion to shareholders through multi-year stock buyback program

"Approximately three years ago, we laid out an ambitious, multi-year strategy to dramatically transform Alexion, position us for the future and drive continued value creation. I am so proud of our tremendous progress advancing and successfully executing on that strategy across the entire organization," said Ludwig Hantson, Ph.D., Chief Executive Officer of Alexion. "As a result, today, Alexion is a very different company than it was in 2017. Our base business is stronger than ever before. Even more importantly, we are in a new stage of company expansion and diversification that provides a path to long-term sustainable growth and allows us to reinvest in innovation for the future and return value to shareholders."

At this year's Virtual Investor Day, Alexion will highlight key portfolio opportunities and drivers of future growth, including:

Robust R&D Portfolio

Alexion's pipeline now includes more than 20 development programs – up from four at the end of 2017 – with the potential for 10 promising launches by 2023. The company continues to expand into additional therapeutic areas as it builds seven rare disease franchises across hematology, nephrology, metabolics, neurology, cardiology, ophthalmology and acute care, which have the potential to deliver more than $10 billion in future peak sales. Despite the challenges posed by COVID-19, Alexion has made significant progress in advancing these programs in 2020.

Sustainability in C5: Leading & Expanding

LEAD: Alexion has already established ULTOMIRIS® (ravulizumab) as the market leader in paroxysmal nocturnal hemoglobinuria (PNH) and is working to make it the new standard of care across the C5 franchise with recent global atypical hemolytic uremic syndrome (aHUS) launches. The company is continuing to innovate to improve the patient experience with both high concentration ULTOMIRIS (100 mg/mL) and once weekly subcutaneous ULTOMIRIS.

EXPAND: Alexion is continuing efforts to expand its C5 franchise into new therapeutic areas. This expansion began with a commitment to neurology, which, in just two years, grew into the company's largest franchise in the U.S., and is on track to quadruple the number of U.S. neurology patients treated by 2025. The company is also working to broaden the reach of ULTOMIRIS with new indications across a variety of therapeutic areas – including through ongoing Phase 3 studies in generalized myasthenia gravis (gMG), neuromyelitis optica spectrum disorder (NMOSD) and amyotrophic lateral sclerosis (ALS) – and is applying its expertise in complement biology with internal patient-driven innovation to develop a third-generation C5 inhibitor, ALXN1720, which was developed with the goal of enabling expansion into new larger rare diseases because of its low-volume subcutaneous administration.

Select Diversification Opportunities in the Portfolio

Alexion has made significant progress diversifying its portfolio beyond C5 over the last several years. Select promising programs from its pipeline – including individual programs and broader platform opportunities – will be highlighted at Investor Day, including:

- **ALXN1840 in Wilson disease:** Alexion is developing a new biomarker to directly quantify the labile bound copper (LBC) in

patients with Wilson disease. As the first direct measure of loosely bound, biologically active copper, this assay could provide a new tool for clinicians to assess copper in the body, helping them more readily diagnose and manage those with the disease. Enrollment is complete in a Phase 3 study of ALXN1840 in Wilson disease, and the data readout remains on track for the first half of 2021.

- **CAEL-101 in AL amyloidosis:** CAEL-101 has the potential to be the first therapy to remove amyloid from tissues and improve organ function, with the aim of prolonging survival. In collaboration with Caelum Biosciences, the Phase 3 program is underway, with the goal of supporting a potential regulatory filing and launch by 2023. Alexion has the option to acquire Caelum based on the Phase 3 trial results.
- **Factor D platform (ALXN2040 & ALXN2050):** Alexion's Factor D platform provides the opportunity to expand treatment for diseases that are currently in the company's portfolio, and to grow into new diseases and therapeutic areas.
    - **ALXN2040:** ALXN2040 has the potential to address clinically evident extravascular hemolysis (EVH) for the small portion of PNH patients on ULTOMIRIS or SOLIRIS® (eculizumab) (<10% of patients) that experience EVH. A Phase 3 study of ALXN2040 as an add-on therapy for these patients is on track to initiate in the fourth quarter of 2020, with topline results expected in the second half of 2022. In addition, Alexion is expanding the ALXN2040 development program with plans to begin a Phase 2 proof-of-concept study in geographic atrophy (GA) in 2021.
    - **ALXN2050:** ALXN2050 has the ability to further build the Factor D platform, first in PNH, where a Phase 2 monotherapy study is ongoing with topline results estimated in the second half of 2021, and secondly, in renal diseases, with a proof-of-concept trial in various renal diseases planned to begin in 2021.
- **Anti-FcRn platform (ALXN1830):** Alexion's anti-FcRn platform has broad applicability across numerous rare IgG-mediated autoimmune diseases. Preliminary PK/PD modeling from the Phase 1 study suggests weekly subcutaneous (SC) injections of 1500mg may have the potential to provide greater than 70 percent IgG lowering. Development of ALXN1830 was paused due to COVID-19, but Alexion plans to reinitiate SC formulation development in early 2021 to complete the Phase 1 healthy volunteer study and to begin Phase 2 studies in warm autoimmune hemolytic anemia (WAIHA) and gMG.

Internal Research & Discovery

Supporting its near-term pipeline, Alexion is continuing to relentlessly pursue innovation and has an unwavering focus on research that will develop solutions to address patient needs. With the company's rare disease capabilities and expertise, in combination with its rebuilt pre-clinical pipeline and expanded areas of focus, Alexion believes it is on track to produce more than five novel INDs by 2025, including two this year.

Capital Allocation Strategy

Since 2017, Alexion has continued to consistently execute from a financial perspective across a number of key areas, driven by strong revenue growth, and expects to achieve a more than 16 percent revenue compound annual growth rate by the end of 2020. In addition, the company has continued to focus on financial discipline to achieve strong operating results and position it for the future. The company will provide updated 2020 financial guidance when it reports third quarter results and expects to raise full-year revenue guidance by more than $200 million. Because of the confidence in its commercial platform, multiple launches in the coming years and a renewed pipeline, Alexion recently announced a new capital allocation strategy that will return value to shareholders, including a commitment to dedicate at least one third of annual free cash flow to share repurchases from 2021 through 2023, for what is expected to be a total of at least $3 billion in stock buybacks covering the four years ending December 2023.

Featured Speakers

The virtual set up of Alexion's Investor Day provides the opportunity to hear from both Alexion executives and scientific leaders, including:

- **David Brennan,** Chairman of the Board
- **Ludwig Hantson,** Ph.D., CEO
- **Aradhana Sarin**, M.D., CFO
- **John Orloff**, M.D., Head of R&D
- **Brian Goff**, Chief Commercial & Global Operations Officer
- **Cristina Quarta**, M.D., Ph.D., CAEL-101 Clinical Development Lead
- **Gianluca Pirozzi**, M.D., Ph.D., Head of Clinical Development & Translational Sciences
- **Anita Hill**, M.D., Ph.D., Hematology Global Medical Affairs Lead
- **Darius Moshfeghi**, M.D., Alexion consultant on Geographic Atrophy, Professor of Ophthalmology & Chief of the Retina Division, Stanford University School of Medicine
- **Sharon Barr,** Ph.D., Head of Research, Bioinformatics & Diagnostics

Investor Day Webcast Information

Alexion will host an audio webcast today from 8:00 a.m. to 12:00 p.m. Eastern Time. The live audio webcast can be accessed here, or from the Investor page of Alexion's website at: http://ir.alexion.com. An archived version of the webcast will also be available through the company's website for a limited time following the event.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialization of life-changing medicines. As a leader in rare diseases for more than 25 years, Alexion has

developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), as well as the first and only approved complement inhibitor to treat anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG) and neuromyelitis optica spectrum disorder (NMOSD). Alexion also has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D) as well as the first and only approved Factor Xa inhibitor reversal agent. In addition, the company is developing several mid-to-late-stage therapies, including a copper-binding agent for Wilson disease, an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases and an oral Factor D inhibitor as well as several early-stage therapies, including one for light chain (AL) amyloidosis, a second oral Factor D inhibitor and a third complement inhibitor. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, metabolic disorders and cardiology. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries. This press release and further information about Alexion can be found at: www.alexion.com.

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Forward-Looking Statement

This press release contains forward-looking statements, including statements related to: anticipated financial results (including short-term guidance and long-range financial guidance), including expected increases to the revenue guidance for 2020, our revenue cumulative average growth rate of at least 10% through 2025 and beyond, and peak revenue from our current pipeline beyond 2025 (and all of the assumptions, judgments and estimates related to such anticipated future results); ambition to quadruple the number of neurology patients in the US by 2025; ambition for 10 product launches by 2023; future plans to continue growing the company's pipeline with more than 5 novel INDs by 2025; plans for additional formulations of ULTOMIRIS (high concentration and subcutaneous) and the timing for regulatory approval and potential benefits of such formulations; anticipated future product launches (and the timing of those launches); that we are in a new stage of company expansion and diversification that provides a path to long-term sustainable growth and allows us to reinvest in innovation for the future and return value to shareholders; the company's capital allocation strategy and plans concerning the repurchase of Alexion shares; the anticipated amount and timing of future share repurchases by the company; plans to make regulatory filings for approval of certain products and product candidates, the expected timing of such filings as well as the expected timing of the receipt of certain regulatory approvals to market a product; the ability of our pipeline and existing products to provide long-term sustainable growth for shareholders; company's plans for future clinical trials and studies, the timing for the commencement and conclusion of future clinical trials and the expected timing of the receipt of results of clinical trials and studies; the company's strategy for long-term value creation; plans to further diversify our assets and establish novel platforms and the benefits of those plans; plans to establish 7 franchises and the targeted indications in each franchise; potential peak sales of our pipeline assets; potential launches of ULTOMIRIS for additional indications and in additional countries, including for ALS; plans and anticipated timing for the development of ALXN1840 in Wilson disease; the development of a new biomarker for Wilson disease to detect labile bound copper and the potential benefits of such biomarker; plans for the development and launch of CAEL-101 as a treatment for AL-Amyloidosis; plans for development and potential indications for ALXN1720; development and commercialization plans for ALXN2040 and ALXN2050, including in PNH, geographic atrophy and renal diseases, and the potential benefits of those therapies; development plans and the potential of ALXN1830; and continued diversification of the pipeline and products. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those forward-looking statements, including for example: our dependence on sales from our C5 products (SOLIRIS and ULTOMIRIS); delays (expected or unexpected) in the time it takes regulatory agencies to review and make determinations on applications for the marketing approval of our products; Alexion's inability to timely submit (or failure to submit) future applications for regulatory approval for our products and product candidates; payer, physician and patient acceptance of ULTOMIRIS as an alternative to SOLIRIS; appropriate pricing for ULTOMIRIS; future competition from biosimilars and novel products; inability to timely initiate (or failure to initiate) and complete future clinical trials due to safety issues, IRB decisions, CMC-related issues, expense or unfavorable results from earlier trials (among other reasons); the number of patients that will use our products and product candidates in the future; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions, anticipated expense or other matters; interruptions or failures in the manufacture and supply of our products and our product candidates; failure to satisfactorily address matters raised by the FDA and other regulatory agencies; results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of our products (or we fail to adequately operate or manage our clinical trials) which could cause us to halt trials, delay or prevent us from making regulatory approval filings or result in denial of regulatory approval of our product candidates; unexpected delays in clinical trials; unexpected concerns that may arise from additional data or analysis obtained during clinical trials; future product improvements may not be realized due to expense or feasibility or other factors; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; inability to complete acquisitions due to failure of regulatory approval or material changes in target or otherwise; inability to complete acquisitions and investments due to increased competition for technology; the possibility that current rates of adoption of our products are not sustained (or anticipated adoption rates are not realized); internal development efforts do not result in commercialization of additional products; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims, lawsuits and challenges against us (including intellectual property lawsuits relating to products brought by third parties against Alexion); the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all; failure to realize the benefits and potential of investments, collaborations, licenses and acquisitions; failure by regulatory authorities to approve transactions; the possibility that expected tax benefits will not be realized or that tax liabilities exceed current expectations; assessment of impact of recent accounting pronouncements; potential declines in sovereign credit ratings or sovereign defaults in countries where we sell our products; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; uncertainties surrounding legal proceedings, company investigations and government investigations; the risk that estimates regarding the number of patients with PNH, aHUS, gMG, NMOSD, HPP and LAL-D and other future indications we are pursuing are inaccurate; the risks of changing foreign exchange rates; risks relating to the potential effects of the company's restructuring; risks related to the acquisition of companies and co-development and collaboration efforts; and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2020 and in our other filings with the SEC. Alexion disclaims any obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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