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The Manton Center at Boston Children's Hospital and Alexion Pharmaceuticals Partner in Artificial Intelligence Approaches to Rare Disease Diagnosis

BOSTON & NEW HAVEN, Conn.--(BUSINESS WIRE)-- The Manton Center for Orphan Disease Research, the Innovation and Digital Health Accelerator at Boston Children's Hospital, and Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) today announced a collaboration to enable rapid and precise diagnoses for patients with suspected rare diseases by combining The Manton Center's expertise in decoding and treating rare diseases with Alexion's genomics and data sciences capabilities.

This press release features multimedia. View the full release here:
<http://www.businesswire.com/news/home/20180227005285/en/>

Under the collaboration, Alexion is sharing its 20 Rare-Disease Questions (20RDQ) platform which integrates a comprehensive landscape of rare disease definitions with clinical feature databases to enable a series of guided questions based upon a patient's medical history and record. When presented with an undiagnosed patient, 20RDQ can help a physician investigate subtle features of the underlying rare disease to converge a diagnosis.

The Manton Center and Innovation Accelerator will combine 20RDQ with internally developed software to create computable descriptions of patients that can be combined with rapid genome sequencing to produce a prioritized list of suspect genetic variants of rare diseases for consideration by a diagnosing physician.

"This partnership combines the unique strengths of each of our organizations to answer critical questions posed by puzzling and difficult diseases," said Alan Beggs, PhD, Director of the Manton Center at Boston Children's Hospital.

"This will accelerate diagnoses and clarify available paths of intervention," added Catherine Brownstein, PhD, Geneticist at Boston Children's and principal investigator of the project.

"Data science approaches hold great promise for addressing the challenge of quickly and accurately diagnosing rare diseases. It is through collaborations such as this that combine expert clinical insight with cutting edge artificial intelligence that have the potential to make a profound difference to patients and their families," said John Reynders, PhD, Vice President of Strategy, Program Management, and Data Sciences at Alexion.

The Manton Center and Alexion will create new sets of computable descriptions of hundreds of diagnosed and undiagnosed patients with rare diseases to train the artificial intelligence engine within 20RDQ to improve diagnosis speed and accuracy while creating actionable insights and disease intervention options in a clinically meaningful timeframe.

About Boston Children's Hospital

[Boston Children's Hospital](#), the primary pediatric teaching affiliate of Harvard Medical School, is home to the world's largest research enterprise based at a pediatric medical center. Its discoveries have benefited both children and adults since 1869. Today, more than 3,000 scientists, including nine members of the National Academy of Sciences, 17 members of the National Academy of Medicine and 11 Howard Hughes Medical Investigators comprise Boston Children's research community. Founded as a 20-bed hospital for children, Boston Children's is now a 415-bed comprehensive center for pediatric and adolescent health care. For more, visit our [Vector](#) and [Thriving](#) blogs and follow us on social media [@BostonChildrens](#), [@BCH_Innovation](#), [Facebook](#) and [YouTube](#).

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development and commercialization of life-changing therapies. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion 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 the leader in complement biology for over 20 years, 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, and metabolic disorders. This press release and further information about Alexion can be found at: www.alexion.com.

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This press release contains forward-looking statements, including statements related to Alexion's diagnostic initiatives. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, 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, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, 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, the possibility that expected tax benefits will not be realized, 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, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PNH, aHUS, gMG, HPP and LAL-D are inaccurate, the risks of changing foreign exchange rates, risks relating to the potential effects of the Company's restructuring and relocation of its corporate headquarters, 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 Annual Report on Form 10-K for the period ended December 31, 2017 and in our other filings with the SEC. Alexion does not intend 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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