Biogen Announces Agreement to Acquire Nightstar Therapeutics to Establish Clinical Pipeline of Gene Therapy Candidates in Ophthalmology

March 4, 2019

Biogen to acquire Nightstar Therapeutics for $25.50 per share

Nightstar Therapeutics is a gene therapy company focused on developing novel treatments for patients suffering from rare inherited retinal diseases that would otherwise lead to blindness

Nightstar has two potentially first-in-class mid- to late-stage clinical assets as well as preclinical programs

Lead asset NSR-REP1 is in Phase 3 development for choroideremia, a rare degenerative disorder that leads to blindness and has no approved treatment options

CAMBRIDGE, Mass., March 04, 2019 (GLOBE NEWSWIRE) -- Biogen (Nasdaq: BIIB) today announced that it has entered into an agreement to acquire Nightstar Therapeutics (NST; Nasdaq: NITE), a clinical-stage gene therapy company based in London, United Kingdom, which is focused on adeno-associated virus (AAV) treatments for inherited retinal disorders. Under the terms of the proposed acquisition, Biogen will pay $25.50 in cash for each NST share. This offer represents a total transaction value of approximately $800 million on a fully diluted basis, after taking into account expected transaction expenses and anticipated cash at closing.

“Ophthalmology is an emerging growth area for Biogen, and we are excited about the opportunity to work with the talented employees at Nightstar to advance potentially transformative gene therapy programs for rare retinal diseases,” said Michel Vounatsos, Biogen’s Chief Executive Officer. “With this proposed acquisition, we are continuing to bolster our pipeline and further execute on our strategy to develop and expand a multi-franchise neuroscience pipeline across complementary modalities. Nightstar would accelerate our entry into ophthalmology by contributing two mid- to late-stage gene therapy assets, with the potential to create long-term shareholder value.”

NST’s lead asset is NSR-REP1 for the treatment of choroideremia (CHM), a rare, degenerative, X-linked inherited retinal disorder, which leads to blindness and has no approved treatments. CHM primarily affects males and is caused by loss of function in the CHM gene which encodes the Rab escort protein-1 (REP-1). The REP-1 protein plays a role in intracellular protein trafficking, and loss of function in the CHM gene leads to abnormal intracellular protein trafficking and impaired elimination of waste products from the retinal pigment epithelium and photoreceptors. Initially, patients with CHM experience poor night vision, and over time progressive visual loss ultimately leads to complete blindness.

NSR-REP1 is comprised of an AAV vector administered by subretinal injection which provides a functioning CHM gene and expression of the REP-1 protein to restore membrane trafficking and thereby slow, stop, or potentially reverse the decline in vision. Data from the Phase 1/2 trial of NSR-REP1 demonstrated potentially meaningful slowing of decline in visual acuity as compared to natural history as well as signs of improved visual acuity in some patients. NSR-REP1 is currently being evaluated in the ongoing Phase 3 STAR trial with data expected in the second half of 2020.

NST’s second clinical program is NSR-RPGR for the treatment of X-linked retinitis pigmentosa (XLRP), which is also a rare inherited retinal disease primarily affecting males with no approved treatments. XLRP is characterized by mutations in the retinitis pigmentosa GTPase regulator (RPGR) gene leading to a lack of active protein transport in photoreceptors. This abnormality leads to loss of the photoreceptor cells, resulting in retinal dysfunction by adolescence and early adulthood, progressing to legal blindness when patients reach their 40s.

NSR-RPGR is comprised of an AAV vector administered by subretinal injection which provides a functioning RPGR gene and thus expression of the RPGR protein, which is critical for protein transport in photoreceptors. The restoration of photoreceptor function is intended to slow, stop, or potentially reverse the decline in vision. Phase 1/2 data from the dose escalation portion of the XIRIUS trial for NSR-RPGR demonstrated an increase in central retinal sensitivity. The Phase 2/3 dose expansion portion of the XIRIUS trial is currently ongoing.

NST’s preclinical pipeline includes NSR-ABCA4 for Stargardt disease and potential programs targeting Best vitelliform macular dystrophy (Best disease) and other genetic forms of retinitis pigmentosa.

The acquisition of NST is planned to be funded through available cash and accounted for as an acquisition of a business. Biogen expects to complete the acquisition by mid-year 2019.

It is intended that the proposed acquisition will be implemented by means of a U.K. Court-sanctioned scheme of arrangement under Part 26 of the U.K. Companies Act 2006. The closing of the proposed acquisition is subject to customary closing conditions, including the approval by NST shareholders, the issuance of an order by the U.K. Court and receipt of regulatory approvals. The proposed acquisition does not require the approval of Biogen’s stockholders.

CONFERENCE CALL AND WEBCAST
On March 4, 2019, at 8:30 a.m. Eastern Time, Biogen will host a live webcast and conference call to discuss the agreement to acquire Nightstar, which will be accessible through the Investors section of Biogen’s website, www.biogen.com. Supplemental information in the form of a slide presentation is also accessible at the same location on the internet and will be subsequently available on the website for at least one month.

Goldman, Sachs & Co. is acting as financial advisor to Biogen, and Ropes & Gray LLP is acting as legal counsel to Biogen.

Centerview Partners is acting as financial advisor to NST, and Skadden, Arps, Slate, Meagher & Flom LLP is acting as legal counsel to NST.

About Biogen
At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases. One of the world’s first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp, and today has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first and only approved treatment for spinal muscular atrophy, and is focused on advancing

neuroscience research programs in Alzheimer's disease and dementia, multiple sclerosis and neuroimmunology, movement disorders, neuromuscular disorders, acute neurology, neurocognitive disorders, pain, and ophthalmology. Biogen also manufactures and commercializes biosimilars of advanced biologics.

We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media – Twitter, LinkedIn, Facebook, YouTube.

Additional Information and Where to Find It
This communication may be deemed to be solicitation material in respect of the proposed acquisition of NST by Biogen. In connection with the proposed acquisition, NST intends to file relevant materials with the Securities and Exchange Commission (SEC), including a proxy statement in preliminary and definitive form. Holders of NST ordinary shares and American Depositary Shares (ADSs) are urged to read all relevant documents filed with the SEC, including NST's definitive proxy statement, because they will contain important information about the proposed acquisition. Investors and security holders are able to obtain the documents (once available) free of charge at the SEC's web site, http://www.sec.gov. Such documents are not currently available.

Participants in Solicitation
Biogen and its directors and executive officers, and NST and its directors and executive officers, may be deemed to be participants in the solicitation of proxies from the holders of NST ordinary shares and ADSs in respect of the proposed acquisition. Information about the directors and executive officers of Biogen and NST is set forth in each company's respective filings with the SEC. Investors may obtain additional information regarding the interest of such participants by reading the proxy statement regarding the proposed acquisition (once available).

Safe Harbor
This press release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, relating to the potential benefits and results that may be achieved through the proposed acquisition; risks and uncertainties associated with drug development and commercialization; the potential benefits, safety, and efficacy of investigational therapies, including NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders; the clinical development program for NSR-REP1 and NSR-RPGR; the anticipated completion and timing of the proposed acquisition; Biogen's strategy and plans; the potential of Biogen's commercial business and pipeline programs, including NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders; and Biogen's capital allocation and investment strategy. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including, without limitation: risks that the proposed acquisition will be completed in a timely manner or at all; the possibility that certain closing conditions to the proposed acquisition will not be satisfied; uncertainty as to whether the anticipated benefits of the proposed acquisition can be achieved; the ability of Biogen to successfully integrate NST's operations and employees; risks of unexpected costs or delays; uncertainty of success in the development and potential commercialization of NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders, which may be impacted by, among other things, the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis; regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of NSR-REP1, NSR-RPGR, NSR-ABCA4, and other potential AAV-based gene-therapies for inherited retinal disorders; Biogen may encounter other unexpected hurdles, which may be impacted by, among other things, the occurrence of adverse safety events, failure to obtain regulatory approvals in certain jurisdictions, or failure to protect intellectual property and other proprietary rights; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; product liability claims; and third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risks factors identified in our most recent annual or quarterly report and in other reports we have filed with the SEC.

These statements are based on our current beliefs and expectations and speak only as of the date of this press release. We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments, or otherwise.

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