Biogen and Alcyone Therapeutics Announce License and Collaboration Agreement to Evaluate a Novel Device to Improve Patient Experience and Access to Neurological ASO Therapies

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- Alcyone's ThecaFlex DRx™ System is an implantable medical device in development for intrathecal drug delivery

CAMBRIDGE, Mass. and LOWELL, Mass., Jan. 04, 2023 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) and Alcyone Therapeutics (Alcyone) have entered into a license and collaboration agreement to develop Alcyone's ThecaFlex DRx™ System, an implantable medical device intended for subcutaneous delivery of antisense oligonucleotide (ASO) therapies into the intrathecal space. Through this agreement, Biogen aims to leverage the ThecaFlex DRx™ System with a goal of improving the patient treatment experience and accessibility for a broader population of people suffering from neurological disorders, such as spinal muscular atrophy (SMA) and amyotrophic lateral sclerosis (ALS).

The ThecaFlex DRx™ System has the potential to be the first implantable device designed to enable routine subcutaneous administration of ASO therapies to the cerebrospinal fluid. The ThecaFlex DRx™ System has received a CE Mark in Europe. In addition, it has also received Breakthrough Device Designation from the U.S. Food and Drug Administration (FDA) and will require further clinical studies before it can be submitted to the FDA for review.

"We are continually listening to the neuromuscular disease community and whenever possible, adapting our work to meet their evolving needs for treatment and patient care," said Priya Singhal, Interim Head of R&D at Biogen. "Biogen looks forward to working with Alcyone to explore the potential of this device, which we believe will provide greater flexibility to people with spinal muscular atrophy and other neurological disorders as well as their doctors in making the right treatment decisions."

"Alcyone designed the ThecaFlex DRx™ System to be a therapeutic delivery alternative for patients with a chronic neurological condition whose current treatment requires repeat lumbar puncture," said PJ Anand, Chief Executive Officer of Alcyone. "This agreement underscores Alcyone's expertise in cerebrospinal fluid delivery technology which we believe will lead to an improved treatment experience for some people living with neurological conditions and their caregivers. We consider Biogen, a global leader, an ideal collaborator toward this mutual goal."

Under the terms of the agreement, Biogen will make an upfront payment of $10 million to Alcyone for an exclusive global license to the ThecaFlex DRx™ System in SMA and ALS as well as a co-exclusive global license in an unnamed indication. Should certain development and commercial milestones be achieved, Alcyone will be eligible to receive up to $41 million in potential milestone payments. The deal also provides flexibility to expand the collaboration as additional ASO therapies progress through Biogen's pipeline.

Biogen and Alcyone will jointly collaborate on clinical development of the ThecaFlex DRx™ System for ASO therapies, and Alcyone will be solely responsible for its manufacture and commercialization. The ThecaFlex DRx™ System will initially be evaluated with SPINRAZA® (nusinersen) in SMA, which will inform pathways for Biogen's broader portfolio of investigational ASO therapies.

About The ThecaFlex DRx™ System
The ThecaFlex DRx™ System (ThecaFlex), a technology within Alcyone's Falcon™ Delivery Platform, is an implantable intrathecal (IT) catheter, catheter fixation device, and subcutaneous port system designed to provide access to the cerebrospinal fluid (CSF) for the infusion of therapy by IT bolus administration. Lumbar puncture (LP), commonly known as a spinal tap, is the current standard of care approach to delivering therapeutics into the CSF. ThecaFlex is designed to be an alternative to LP, especially for people with challenging anatomy or for those who require multiple anesthesia and radiation exposures for repeat LPs.

The ThecaFlex DRx™ System has received CE Mark in Europe and Breakthrough Device Designation from the U.S. Food and Drug Administration (FDA). ThecaFlex is not approved by the FDA. For more information, visit www.alcyonetx.com.

About SPINRAZA® (nusinersen)
SPINRAZA is approved to treat infants, children and adults with spinal muscular atrophy (SMA) and is approved in more than 60 countries. As a foundation of care in SMA, more than 13,000 individuals have been treated with SPINRAZA worldwide.[1]

SPINRAZA is an antisense oligonucleotide (ASO) that targets the root cause of SMA by continuously increasing the amount of full-length survival motor neuron (SMN) protein produced in the body.[2] It is administered directly into the central nervous system, where motor neurons reside, to deliver treatment where the disease starts.[2]

SPINRAZA has demonstrated sustained efficacy across ages and SMA types with a well-established safety profile based on data in patients treated up to 8 years, combined with unsurpassed real-world experience.[3] The nusinersen clinical development program encompasses more than 10 clinical studies, which have included more than 460 individuals across a broad spectrum of patient populations, including two randomized controlled studies (ENDEAR and CHERISH). The ongoing SHINE and NURTURE open-label extension studies are evaluating the long-term impact of SPINRAZA. The most common adverse events observed in clinical studies were respiratory infection, fever, constipation, headache, vomiting and back pain. Laboratory tests can monitor for renal toxicity and coagulation abnormalities, including acute severe low platelet counts, which have been observed after administration of some ASOs.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals, Inc. (Nasdaq: IONS). Please click here for Important Safety Information and full Prescribing Information for SPINRAZA in the U.S., or visit your respective country's product website.

About Biogen
As pioneers in neuroscience, Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles

Weissmann, Heinz Schaller, Sir Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today, Biogen has a leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, and developed the first and only approved treatment to address a defining pathology of Alzheimer's disease. Biogen is also commercializing biosimilars and focusing on advancing one of the industry's most diversified pipelines in neuroscience that will transform the standard of care for patients in several areas of high unmet need.

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.

About Alcyone Therapeutics
Alcyone Therapeutics is a biotechnology company pioneering next-generation precision gene-based therapies for complex neurological conditions. The company integrates innovation in neuroscience, precision dosing platforms, and manufacturing capabilities to deliver transformative therapies to patients. Alcyone leverages the synergy between Falcon™, the Company's proprietary intrathecal precision dosing and biodistribution platform that incorporates deep knowledge of cerebral spinal fluid (CSF) dynamics, computational modeling, and bioengineering, and novel gene-based therapeutics platforms developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital (AWRI). This comprehensive approach allows for the optimization of central nervous system (CNS) dosing and delivery to better target the pathophysiology and anatomy specific to various neurological diseases. Alcyone's lead programs utilize X chromosome reactivation for X-linked disorders and targets the treatment of Rett syndrome, and gene replacement for the treatment of IGHMBP2-related disorders including spinal muscular atrophy with respiratory distress type 1 (SMARD1) and Charcot Marie Tooth disease type 2S (CMT2S). For more information, visit www.alcyonetx.com.

Biogen Safe Harbor
This news release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, the potential benefits, safety and efficacy of the ThecaFlex DRx™ System; the clinical development program for ThecaFlex DRx™ system; our collaboration with Alcyone; the potential of our commercial business and pipeline programs and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "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, uncertainty of success in the development and potential commercialization of the ThecaFlex DRx™ System; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates; the occurrence of adverse safety events; the risks of unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; and the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, results of operations and financial condition. 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 risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this news 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.

References:

1. Based on commercial patients, early access patients, and clinical trial participants through March 31, 2022.
2. SPINRAZA U.S. Prescribing Information. Available at: https://www.spinraza.com/content/dam/commercial/specialty/spinraza/caregiver/en_us/pdf/spinraza-prescribing-information.pdf. Accessed: October 2022.
3. Core Data sheet, Version 13, October 2021. SPINRAZA. Biogen Inc, Cambridge, MA.

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