Biogen Idec and Knopp Neurosciences Announce License Agreement for Late-Stage ALS Drug Candidate

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- Biogen Idec expects to initiate Phase 3 program in the first half of 2011
- Equity investment and potential license payments of $345 million

WESTON, Mass. & PITTSBURGH--(BUSINESS WIRE)--Biogen Idec (NASDAQ: BIIB) and Knopp Neurosciences today announced they have entered into an exclusive, worldwide license agreement under which Biogen Idec will develop and commercialize KNS-760704 (dexpramipexole) for the treatment of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, and potentially other indications.

KNS-760704 is a novel oral neuroprotective therapy under development. In a Phase 2 study of ALS patients conducted by Knopp, the compound achieved its primary endpoint evaluating safety and tolerability and showed favorable dose-related effects in preserving motor function and extending survival. KNS-760704 has received orphan drug designation from the U.S. Food and Drug Administration and the European Commission for the treatment of patients with ALS, as well as Fast Track designation from the FDA. Biogen Idec expects to initiate a Phase 3 program of the compound in the first half of 2011.

Under the terms of the agreement, Biogen Idec will lead the development of KNS-760704 for ALS and its potential commercialization in global markets, with Knopp providing development support and conducting certain U.S. commercialization activities under the direction of Biogen Idec. As part of the transaction, Biogen Idec will purchase $60 million of Knopp stock, provide an up-front payment of $20 million and additional payments of up to $265 million based on the achievement of development, regulatory, and sales milestones. Biogen Idec will also pay tiered, double-digit royalties to Knopp on worldwide sales.

"We are very pleased to enter into this agreement with Biogen Idec, a world leader in neurology drug development and commercialization," said Michael Bozik, M.D., president and CEO of Knopp, a privately held, Pittsburgh-based biopharmaceutical company. "Biogen Idec's proven track record of delivering innovative medicines for diseases with high unmet needs makes it the ideal partner to advance KNS-760704 in ALS."

"We are excited to begin this new collaboration with Knopp on KNS-760704, a potential treatment for ALS with promising Phase 2 data," said George Scangos, Ph.D., CEO of Biogen Idec. "ALS is a devastating disorder and, with only one approved therapy, there is a tremendous need to provide more therapeutic options for patients. This disease is a natural fit for Biogen Idec, given our global capabilities in neurology, and we look forward to beginning a Phase 3 program."

Knopp has completed a two-part Phase 2 study that assessed the effects of KNS-760704 in the same subjects over two randomized, double-blind treatment periods separated by a one-month washout. These Phase 2 data were presented at the 20th International Symposium on ALS/MND in Berlin in December 2009. In Part 1 of the study, 102 subjects received daily doses of 50 mg, 150 mg, or 300 mg of KNS-760704 or placebo for 12 weeks. KNS-760704 showed a dose-dependent trend in slowing the rate of disease progression, as measured by the difference in slopes of the ALS Functional Rating Scale-Revised (ALSFRS-R) across treatment groups, with the greatest benefit observed in the 300 mg dose group.

In Part 2 of the study, 92 subjects were re-randomized to receive daily doses of 50 mg or 300 mg of KNS-760704 for 24 weeks. In addition to results again suggesting a dose-dependent trend in slowing the rate of disease progression as measured by the ALSFRS-R, there was also a trend toward a survival benefit in the 300 mg group compared with the 50 mg group. In an exploratory test comparing subject rankings on the basis of mortality and functional outcomes, subjects in the 300 mg group had a significantly improved outcome compared with the 50 mg group.

KNS-760704 was generally well tolerated. There were no per-treatment group differences in the frequency of adverse events or serious adverse events reported in the Phase 2 trial. The most common adverse events reported more frequently than in placebo subjects were fall, nausea, and joint pain. Some cases of neutropenia were observed, which resolved when therapy was discontinued and generally did not recur when therapy was reinitiated.

Clearview Projects of Princeton, N.J., and Wolpert Associates Inc. of Summit, N.J., acted as transaction advisors to Knopp. K&L Gates LLP acted as Knopp's corporate/licensing counsel and Pepper Hamilton LLP as its patent counsel.

Supplemental information in the form of a slide presentation will be posted and accessible on the Investors section of www.biogenidec.com at the time of publication of this press release and will be available through September 30, 2010.

About KNS-760704 (dexpramipexole)

KNS-760704 is the chirally pure form of dexpramipexole, a low molecular weight benzothiazole shown to improve mitochondrial function and to confer significant cellular protection in neurons under stress. KNS-760704 is highly orally bioavailable, water soluble, renally excreted, and only moderately protein bound. KNS-760704 was originally identified as a candidate therapy for ALS by James Bennett, M.D., Ph.D., then of the University of Virginia. Certain rights licensed by Knopp to Biogen Idec include rights licensed from the University of Virginia Patent Foundation (www.uvapf.org). The compound has received orphan drug designation from the U.S. Food and Drug Administration and the European Commission for the treatment of patients with ALS, as well as Fast Track designation from the FDA.

About ALS

Amyotrophic lateral sclerosis, also known as Lou Gehrig's disease and motor neuron disease, is a universally and rapidly fatal neurodegenerative disorder characterized by progressive muscle weakness and wasting. ALS affects adults in the prime of life and creates a substantial burden for caregivers. U.S. prevalence is approximately 20,000 and the global incidence is approximately two per 100,000. Only one drug has been approved for the treatment of ALS. Life expectancy after symptom onset is usually three to five years.

About Biogen Idec

Biogen Idec creates new standards of care in therapeutic areas with high unmet medical needs. Founded in 1978, Biogen Idec is a global leader in the discovery, development, manufacturing, and commercialization of innovative therapies. Patients worldwide benefit from Biogen Idec's significant products that address diseases such as lymphoma, multiple sclerosis, and rheumatoid arthritis. For product labeling, press releases and additional information about the company, please visit www.biogenidec.com.

About Knopp Neurosciences

Knopp Neurosciences Inc., based in Pittsburgh, PA, USA, is a drug discovery and development company focused on delivering breakthrough treatments for neurological disorders through innovation, experience, and partnership. The company's lead product candidate is KNS-760704, an orally bioavailable small molecule in development for the treatment of ALS. Knopp's leadership includes experienced neuroscience drug development and discovery executives formerly associated with major pharmaceutical companies. Knopp's financing has been led by Saturn Capital Inc. of Boston as placement agent and Saturn Partners II as lead funder. Funders also include co-founder LaunchCyte LLC, Innovation Works, and Kramer Capital Partners.

Safe Harbor/Forward-Looking Statements

This press release contains "forward-looking statements," including statements relating to planned regulatory filings and clinical development programs. All forward-looking statements are based on the companies' current assumptions and expectations and involve risks, uncertainties and other important factors, specifically including the uncertainties inherent in clinical trials and product development programs, the availability of funding to support continued research and studies, the availability or potential availability of alternative therapies or treatments, the availability of patent protection for the discoveries, and strategic alliances, as well as additional factors that may cause actual results to differ from the companies' expectations, including those set forth in the Risk Factors section of Biogen Idec's most recent annual or quarterly report filed with the SEC. There can be no assurance that KNS-760704 will be successfully developed or manufactured or that final results of clinical studies will be supportive of regulatory approvals required to market the products. The companies undertake no obligation to update or revise any such forward-looking statements, whether as a result of new information, future events, or otherwise.

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