Biogen Idec and Knopp Biosciences Announce Enrollment of the First Patient in a Global Phase III Study of Dexpramipexole for ALS

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-- Start of Phase III Trial for Promising Compound Brings Excitement to ALS Community --

WESTON, Mass. & PITTSBURGH--(BUSINESS WIRE)--Biogen Idec (NASDAQ: BIIB) and Knopp Biosciences today announced enrollment of the first patient in EMPOWER, a multi-national Phase III study evaluating the efficacy, safety and pharmacokinetics of dexpramipexole in patients with amyotrophic lateral sclerosis (ALS). ALS, also known as Lou Gehrig's disease and motor neuron disease (MND), is a rapidly progressive and degenerative disease of motor neurons in the brain and spinal cord. It causes muscle atrophy and spasticity, resulting in weakness, progressive paralysis and, ultimately, death usually by respiratory failure.

"ALS is a serious disorder that affects patients in the prime of life and for which there is an urgent need for new and effective treatments," said Merit Cudkowicz, M.D., MSc, Director of the MDA ALS Clinic at Massachusetts General Hospital and Principal Investigator in the EMPOWER study. "In the past several years, there has been an explosion of research in ALS that has helped us better understand the disease and potentially paves the way for the discovery and development of new treatments for ALS."

EMPOWER is a Phase III, randomized, double-blind, placebo-controlled, parallel-group, multi-center study that will assess the safety and efficacy of dexpramipexole in people with familial or sporadic ALS. Approximately 804 patients will be randomized in a one-to-one ratio to receive either dexpramipexole 150 mg twice daily or placebo and will be followed for a period of at least 12 months. Investigators also have the option to maintain treatment with the current standard-of-care in ALS for all patients enrolled in the study. The primary objective will be assessed using a joint ranking of functional outcomes adjusted for mortality on the ALS Functional Rating Scale, Revised (ALSFRS-R), a validated rating instrument used by physicians for monitoring the progression of disability in patients with ALS.

"Biogen Idec is committed to working with the ALS community to find new treatment options for this deadly disease and to improve the lives of people with ALS," said Alfred Sandrock, M.D., Ph.D., Senior Vice President of Development at Biogen Idec. "Based on promising data from earlier clinical trials, we believe that dexpramipexole has the potential to be a significant advance for people suffering from ALS, and we will fully explore its potential as a new treatment for this devastating disease."

"Knopp Biosciences is very pleased to see dexpramipexole entering Phase III development with the initiation of the EMPOWER study," said Michael Bozik, M.D., President and CEO of Knopp. "The promise of dexpramipexole has brought together a motivated multi-national network of investigators and study coordinators committed to determining if dexpramipexole can fulfill its potential as a treatment for patients with ALS."

Dexpramipexole was well-tolerated in three Phase I studies in healthy volunteers who received dexpramipexole in single doses up to 300 mg or multiple doses of up to 150 mg twice daily for four-and-a-half days. Dexpramipexole was also well-tolerated in a two-part Phase II study (CL201) that evaluated about 100 ALS patients treated for up to nine months and in a subsequent Phase II extension study (CL211) that has continued to follow ALS patients for about two additional years. In the first part of CL201, dexpramipexole at 150 mg twice daily showed a trend toward slowing functional decline over a 12-week period compared to placebo. Following re-randomization in the second part of CL201, dexpramipexole at 150 mg twice daily again showed a trend toward slowing functional decline and also showed a trend toward improving survival compared to low dose over a six-month period. In CL201, the joint ranking, which incorporates both function and survival and which is the primary endpoint of the Phase III trial, significantly favored dexpramipexole 150 mg twice daily compared to 25 mg twice daily.

Biogen Idec has agreed with the U.S. Food and Drug Administration (FDA) on a Special Protocol Assessment (SPA) for the design of the Phase III clinical trial of dexpramipexole. Under an SPA, the FDA evaluates a clinical trial protocol to assess whether it is adequate to meet current scientific and regulatory requirements for a potential application to market a new drug.

The first patient dosed in the EMPOWER study triggers a $10 million milestone payment from Biogen Idec to Knopp.

About Dexpramipexole

Dexpramipexole is a novel, orally administered compound under development for the treatment of ALS. It has shown neuroprotective properties in multiple in vitro and in vivo studies and may work by increasing the efficiency of mitochondria, the energy producing portion of the body's cells. Mitochondria in the motor neurons undergo significant stress in ALS patients. In a Phase II study, dexpramipexole achieved its primary objective evaluating safety and tolerability and also showed a trend toward dose-related slowing of functional decline and a trend toward extending survival at the highest dose (150 mg twice daily). Dexpramipexole has been granted Fast Track status by the FDA, which may result in an expedited review, and has received orphan drug designation for the treatment of ALS from both the FDA and the European Medicines Agency.

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. Worldwide incidence of ALS is approximately two people per 100,000. Only one drug has been approved for the treatment of ALS, and it typically extends survival by two to three months. Life expectancy after the onset of symptoms is usually three to five years.

About Biogen Idec

Biogen Idec uses cutting-edge science to discover, develop, manufacture and market biological products for the treatment of serious diseases with a focus on neurological disorders. Founded in 1978, Biogen Idec is the world's oldest independent biotechnology company. Patients worldwide benefit from its leading multiple sclerosis therapies, and the company generates more than $4 billion in annual revenues. For product labeling, press releases and additional information about the company, please visit www.biogenidec.com.

About Knopp Biosciences LLC

Knopp Biosciences LLC, based in Pittsburgh, PA, is a drug discovery and development company focused on delivering breakthrough treatments for unmet medical needs through innovation, experience, and partnership. The company’s lead product candidate is KNS-760704 (dexpramipexole), an orally bioavailable small molecule in development for the treatment of ALS and potentially other indications under a license agreement with Biogen Idec. Knopp's current discovery efforts are directed to pharmacological mediation of mitochondrial and ion channel disease targets. 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 includes forward-looking statements, including statements about the development of dexpramipexole in ALS. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "expect," "forecast," "intend," "may," "plan," "will" and other words and terms of similar meaning. You should not place undue reliance on these statements.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including our dependence on our three principal products, AVONEX®, RITUXAN® and TYSABRI®, the importance of TYSABRI's sales growth, product competition, uncertainty of success in commercializing other products, the occurrence of adverse safety events with our products, changes in the availability of reimbursement for our products, adverse market and economic conditions, problems with our manufacturing processes and our reliance on third parties, our dependence on collaborations over which we may not always have full control, failure to execute our growth initiatives, failure to comply with government regulation and possible adverse impact of changes in such regulation, charges and other costs relating to our properties, fluctuations in our effective tax rate, our ability to attract and retain qualified personnel, the risks of doing business internationally, our ability to protect our intellectual property rights and the cost of doing so, product liability claims, fluctuations in our operating results, the market, interest and credit risks associated with our portfolio of marketable securities, our level of indebtedness, environmental risks, aspects of our corporate governance and collaborations, representation of activist shareholders on our board of directors, and the other risks and uncertainties that are described in the Risk Factors section of 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.

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