Edimer Initiates Phase 2 Trial of EDI200 in XLHED-Affected Male Newborns

-- First neonate subject with X-linked hypohidrotic ectodermal dysplasia completes dosing with the novel ectodysplasin replacement protein EDI200 --

Cambridge, Mass. – October 8th, 2013 – Edimer Pharmaceuticals, a biotechnology company focused on developing an innovative therapy for the rare genetic disorder X-linked Hypohidrotic Ectodermal Dysplasia (XLHED), today announced the enrollment and completed dosing of the first XLHED-affected neonate in a Phase 2 trial of EDI200, the company's novel, proprietary, recombinant protein. XLHED is an ultra-rare orphan disease of ectoderm development associated with a lack of sweat glands, poor temperature control, respiratory problems, and hair and tooth malformations. Affected individuals are at risk for serious and potentially life-threatening hyperthermia and respiratory infections. EDI200 replaces EDA-A1, the protein missing in XLHED and a key regulator of skin and tooth development. If fully developed and approved, EDI200 will be the first protein therapeutic to provide a sustained correction of the symptoms of this disorder.

"The completed dosing of the first patient in the neonate study of EDI200 represents a significant milestone for Edimer and those affected with XLHED," said Neil Kirby, Ph.D., President and CEO of Edimer. "Today is the culmination of several years of dedicated and impassioned work by the Edimer team and our external collaborators. We celebrate the courage of conviction that supported the science at the foundation of EDI200's development and the selfless participation of those involved in the clinical trials who share our goal to create a clinically-significant, life-long health benefit for those affected with XLHED."

"The first research project the NFED funded was in 1989 for the gene identification of X-Linked Hypohidrotic Ectodermal Dysplasia, XLHED," said Anil Vora, President of the Board of Directors at the National Foundation for Ectodermal Dysplasias (NFED). "On that day, we started an amazing journey that lead to the development of EDI200. Along the way, NFED families stepped up to volunteer for every research project in the battle to find a cure. We are thrilled to have witnessed and supported the ongoing development of EDI200 and look forward to learning the outcomes of this clinical study."

About the Phase 2 Clinical Trial

The Phase 2 clinical trial is designed to evaluate the safety, pharmacokinetics, pharmacodynamics and efficacy of EDI200 in XLHED-affected male newborns in the first two weeks of life. EDI200 dosing will be initiated between the 2nd and 14th days of life, with each study subject receiving two doses per week for a total of five doses. For additional information on this clinical trial, please visit clinicaltrials.gov, identifier NCT01775462.

Phase 1 Clinical Trial Outcomes

The EDI200 Phase 1 trial was an open-label, multicenter study to evaluate the safety and pharmacokinetics of EDI200. XLHED-affected males and females were enrolled in anticipation of future studies dosing XLHED-affected neonates. Six adult subjects, four males and two females, were enrolled at two U.S. sites and all successfully completed the five dose course of EDI200 over two weeks. EDI200 was generally well tolerated at the doses to be studied in neonatal subjects. No SAE's

were reported and the majority of AEs in this open-label study were mild and transient with full resolution. An independent Data Safety Monitoring Board reviewed all adult safety data and approved the dosing and monitoring protocol for the Phase 2 neonate study. For a developmental disorder such as XLHED, there was no expectation of clinical benefit following EDI200 administration to adult subjects. However, several subjects administered the higher dose of EDI200 demonstrated a short-term improvement in hair growth, dry eye symptoms and lung inflammation. Larger studies and a longer follow-up period will be required to determine if these changes represent an unanticipated and possibly sustainable benefit related to EDI200 treatment.

About EDI200

EDI200 is an ectodysplasin-A1 (EDA-A1) replacement protein, representing the first of a new class of molecules rationally designed to correct a specific developmental disorder. EDI200 has been shown to bind specifically to the EDA-A1 receptor (EDAR), activating the signaling pathways that lead to normal ectoderm development. EDI200 has demonstrated substantial and durable efficacy in animal models of XLHED with notable reduction in mortality and morbidity. The U.S. Food and Drug Administration (FDA) granted Orphan Drug designation and Fast Track status to EDI200. EDI200 also has Orphan Drug designation in Europe.

About XLHED

XLHED (also known as Christ-Siemens-Touraine Syndrome) is a rare disorder of development resulting from genetic mutations in the ectodysplasin gene (EDA). Patients affected by XLHED are at risk for life-threatening hyperthermia based on their inability to regulate body temperature, and for clinically-significant pneumonias resulting from their abnormality in respiratory secretions. Cardinal signs and symptoms in XLHED include diminished/absent sweat, reduced and abnormal airway secretions, few and often misshapen teeth, and absent or early hair loss from face and scalp.

XLHED patients surviving infancy are predisposed to atopy presenting with eczema and asthma, chronic sinusitis, recurrent nose bleeds, and dry eye complications. Almost uniformly they require dental interventions including early prostheses and later implants. Their susceptibility to hyperthermia, may impact normal participation in outdoor activities, sports and school attendance. Both medical and self-esteem issues are life-long in this disorder. As is generally true with X-linked inheritance, males are fully affected while females are variably affected.

About Edimer Pharmaceuticals

Edimer is a privately held biotechnology company based in Cambridge, Massachusetts dedicated to delivering a significant and durable improvement in the health and quality of life for future generations affected by XLHED. Edimer was established in 2009 with investment from Third Rock Ventures and VI Partners. NEA and Sanofi-Genzyme BioVentures joined the initial investors in a Series B round of equity financing that closed in July of 2013.

For further information on Edimer Pharmaceuticals, please visit www.edimerpharma.com. To receive regular updates about Edimer Pharmaceuticals' progress please join the XLHED network at www.xlhednetwork.com.

CONTACTS:

Edimer Pharmaceuticals
Neil Kirby, Ph.D, President & CEO
617-758-4301

Maureen L. Suda (Media)
Suda Communications LLC
585-387-9248

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