Edimer Pharmaceuticals' EDI200 Expands to Higher Dose Cohort in Phase 2 Trial Following Positive Review of Safety Data

-- Company Initiates Enrollment of First XLHED Natural History Study to Characterize Progression of Untreated Disease and Related Phenotype --

Cambridge, Mass. – May 28, 2014 – Edimer Pharmaceuticals, a biotechnology company focused on developing an innovative therapy for the rare genetic disorder, X-linked Hypohidrotic Ectodermal Dysplasia (XLHED), today announced completion of dosing of the first subject in the second cohort of the ongoing Phase 2 clinical trial. Newborns in cohort 2 are administered the company's proprietary ectodysplasin replacement protein EDI200 at the dose (10 mg/kg) that was found to maximize response and health benefits in the preclinical model of XLHED. Prior to cohort 2 initiation, the independent Data Safety Monitoring Board (DSMB) reviewed safety data from cohort 1 neonates dosed at 3 mg/kg and approved the dose escalation. 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. Following successful completion of a Phase 1 study of EDI200 in XLHED-affected adults, the Phase 2 clinical trial of EDI200 in XLHED-affected newborn male subjects is being conducted at several European and three U.S. medical centers. EDI200 dosing is initiated between the 2nd and 14th days of life, with each study subject receiving 2 doses per week for a total of 5 doses. Edimer expects the trial to be fully enrolled by the fourth quarter of 2014, with top-line data expected in the first half of 2015. If fully developed and approved, EDI200 will be the first protein therapeutic to provide a sustained correction of the symptoms of this disorder.

"We remain grateful to the families affected by XLHED – their encouraging words of support and active involvement in all stages of the EDI200 clinical development effort has been nothing short of incredible," said Neil Kirby, Ph.D., President and CEO of Edimer. "Their unyielding collaboration will be the fundamental element to our goal of completing enrollment of the clinical trial later this year."

As part of Edimer's clinical development plan, the company also recently began enrollment of a natural history study which will enroll male patients, ages 36 months and younger, who have a diagnosis of XLHED based on genetic testing and who have not received an investigational study drug. This study will include collection of all relevant medical history and documentation of clinical outcomes using age appropriate, minimally invasive technologies. Data will be collected both retrospectively, back to pregnancy assessments that may be available, and prospectively through age 5 years. The natural history study is intended to provide new and clinically - predictive information for the benefit of patients, families, and health care providers, in addition to clinical investigators and regulatory agencies assessing trials for therapeutic interventions.

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.

EDI200 is currently being tested in a phase 2 clinical trial designed to evaluate the safety, pharmacokinetics, pharmacodynamics and efficacy of EDI200 in XLHED-affected male newborns in the first two weeks of life. For additional information on this clinical trial, please visit clinicaltrials.gov, identifier NCT01775462.

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. For further information on Edimer Pharmaceuticals, please visit www.edimerpharma.com.

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