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**FOR IMMEDIATE RELEASE:**

**Ultragenyx Announces Three Abstracts Accepted for Poster Presentation at 17<sup>th</sup>  
Annual World Muscle Society Congress**

**NOVATO, CA – October 10, 2012** - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced that three abstracts related to UX001 Sialic Acid Extended-Release for Hereditary Inclusion Body Myopathy were accepted for poster presentation at the 17<sup>th</sup> Annual World Muscle Society Congress October 9-12, 2012 in Perth, Australia.

The following abstracts have been presented as a poster:

- UX001-CL101: A Phase 1 Study To Evaluate The Safety And Pharmacokinetics Of Single And Repeat Doses Of Sialic Acid Extended-Release (SA-ER) Tablets In Patients With Hereditary Inclusion Body Myopathy (HIBM)
- Results From A Pilot Study Of Muscle Strength And Function In Adults With Hereditary Inclusion Body Myopathy (HIBM)
- The HIBM Patient Monitoring Program (HIBM-PMP): A Registry And Natural History Study To Advance Research And Clinical Management In Hereditary Inclusion Body Myopathy (HIBM)

These abstracts are available on the Ultragenyx website, [www.ultragenyx.com](http://www.ultragenyx.com).

**About HIBM**

HIBM is also known as GNE myopathy, Quadriceps Sparing Myopathy (QSM), Inclusion Body Myopathy type 2, Distal Myopathy with Rimmed Vacuoles (DMRV) and Nonaka myopathy. HIBM is a severe, adult-onset, progressive, genetic neuromuscular disease caused by a deficiency of an enzyme in the first step of sialic acid biosynthesis needed for the modification of proteins and fats. Patients with HIBM typically begin to have weakness and abnormal walking at 18 to 30 years of age. Over the ensuing 10 to 20 years, many patients progressively lose significant functional ability and become wheelchair-bound. There are no approved treatments for this disease.

**About Ultragenyx**

*Transforming good science into great medicine for rare genetic diseases*



Ultragenyx is a privately held, developmental stage biotechnology company committed to bringing life-enhancing therapeutics for patients with rare and ultra-rare genetic diseases, also known as orphan and ultra-orphan diseases, to market. The company focuses on rare metabolic diseases that affect small numbers of patients, but for which the unmet medical need is high and there are no effective treatments. Ultragenyx intends to build a sustainable pipeline of safe and effective therapies to address these underserved diseases. Ultragenyx' lead program, UX001, is being evaluated as a potential treatment for hereditary inclusion body myopathy (HIBM), also known as GNE myopathy. The UX001 program has been granted orphan drug designation in the US and the EU.

The company is led by an experienced management team in rare disease therapeutics. Ultragenyx is striving toward an improved model for successful rare disease drug development, which has the potential to increase efficiency while maintaining appropriate safety and efficacy standards. The company believes that it can deliver significant value to patients by building a high-quality pipeline of rare disease therapeutics and efficiently transforming good science into great medicine.

For more information on Ultragenyx, please visit the company's website at [www.ultragenyx.com](http://www.ultragenyx.com).

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