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## **EMBARGOED FOR RELEASE:**

Thursday, May 19, 2011  
Contact: Mary Dean ([mdean@asgct.org](mailto:mdean@asgct.org))  
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## **ASGCT 14<sup>th</sup> Annual Meeting: Gene therapy may be used to treat muscular dystrophy**

**SEATTLE (Thursday, May 19, 2011)** – A gene therapy study using a modified virus vector containing a protein to treat muscular dystrophy showed promising results, according to findings presented today at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting in Seattle.

Muscular dystrophy is an inherited highly debilitating muscle disease that comes in several forms. Limb Girdle Muscular Dystrophy (LGMD 2C) is characterized by progressive muscle weakness in the hips, shoulders, and legs, leading to the inability to walk by preadolescence. LGMD 2C is caused by a deficiency in the gamma sarcoglycan gene (SGC), which results in the muscles being unable to form the proteins needed for normal muscle function. At this time there is no known treatment or cure for this disease.

Serge Herson, MD of Hôpital Pitié-Salpêtrière, Assistance Publique – Hôpitaux de Paris, France, and colleagues, treated a group of nine patients suffering from LGMD, with a modified virus vector carrying the SGC gene. Researchers found that no adverse effects occurred and in muscle biopsies taken 30 days after the injections, SGC protein was detected. Researchers concluded that this study paves the way for future developments in gene therapy of hereditary muscle diseases. The clinical study was sponsored by Genethon (Evry, France) and funded by AFM (Association Française contre les Myopathies)

### **Abstract:** 51

**Title:** A Phase I Dose-Escalating Study of AAV1 –  $\gamma$ -Sarcoglycan Gene Therapy for Limb Girdle Muscular Dystrophy Type 2C

**Date:** Thursday, May 19, 2011

**Session Info:** Presidential Symposium (8 am – 10:15 am)

**Presentation Time:** 9:25 am

**Room:** Washington State Convention Center, Room 6BC

The American Society of Gene & Cell Therapy (ASGCT) is a professional non-profit medical and scientific organization dedicated to the understanding, development and application of genetic and cellular therapies and the promotion of professional and public education in the field. For more information on ASGCT, visit its website, [www.asgct.org](http://www.asgct.org).

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