

Basel, Switzerland, and Montreal, Canada – 29 July 2014

## **Roche and AmorChem announce alliance focused on myotonic muscular dystrophy 1**

Roche (SIX: RO, ROG; OTCQX: RHHBY) and AmorChem L.P., a Montreal-based venture capital firm investing in life science projects, today announced they have entered into a collaboration to discover novel small molecule disease-modifying therapy for the treatment of myotonic muscular dystrophy 1, or Steinert's disease. Myotonic dystrophy is a progressive degenerative disease that affects an estimated 130,000 people in US, EU and Japan. There is currently no approved treatment available to slow or stop disease progression.

The collaboration will focus on the development of novel small molecules capable of correcting the consequences of the splicing deficit caused by the myotonic dystrophy 1 gene mutation. Through this approach, some of the molecular alterations caused by the disease process may be corrected and the progression of disease may be curtailed. The enabling technology was developed by Dr. Pascal Chartrand, a principal investigator at University of Montreal, and licensed to AmorChem by Univalor, the university's technology transfer group. Discovery will take place at AmorChem's medicinal chemistry incubator, NuChem Therapeutics, and in Dr. Chartrand's laboratory. Roche will provide scientific support and will contribute R&D funding together with AmorChem.

"By targeting the molecular consequences of the genetic mutation that causes myotonic dystrophy, we aim to slow down or stop the progression of this currently untreatable, chronic and slowly progressing muscle-wasting disease," said Luca Santarelli, Global Head of Neuroscience, Ophthalmology and Rare Diseases at Roche Pharma Research and Early Development. "The partnership with AmorChem fits well into our discovery externalization strategy, which aims to leverage external scientific excellence and experienced entrepreneurs to complement our internal portfolio of innovative drug programs."

"We are very pleased to be working together with Roche to pursue this project. Not only is it a testament to the value of our ongoing collaboration with Dr. Chartrand and his team, but it also highlights the contributions made by our affiliate, NuChem Therapeutics," said Inès Holzbaur, general partner at AmorChem. "We look forward to continuing the development of this technology together with Roche, and are hopeful that one day it will impact the lives of patients suffering from this disease."

“AmorChem was founded on the premise that there is considerable opportunity in the high quality innovation prevalent in Quebec-based universities, and that the pharmaceutical industry would find significant worth in our early-stage projects. This new partnership with Roche indeed delivers on that proposition and emphasizes the value being created by our alliances with Univalor and the University of Montreal,” said Elizabeth Douville, general partner at AmorChem.

Under the terms of the agreement, Roche will have the option to acquire an exclusive, worldwide license at the end of the collaboration. AmorChem may earn up to \$107 million in total, based on developmental and commercial milestones, and single-digit tiered royalties.

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### **About myotonic dystrophy 1 / Steinert’s disease**

Myotonic dystrophy 1 is a rare genetic disease caused by a triple-repeat mutation in the DMPK gene on chromosome 19. Patients with myotonic dystrophy 1 may experience varying ages of disease onset as well as varying disease severity. Most common effects of disease are manifested in skeletal muscle atrophy, but symptoms also include cognitive, cardiac, respiratory and gastrointestinal dysfunctions. As a progressive degenerative disease that affects an estimated 130,000 patients in US, EU and Japan, there is currently no approved treatment available to slow or stop disease progression.

### **About AmorChem**

AmorChem L.P. ([www.amorchem.com](http://www.amorchem.com)) is a venture capital fund located in Montreal focused on investing in promising life science projects originating from Quebec-based universities and research centres. The principal limited partners of this fund are Investissement-Québec, FIER Partenaires, Fonds de solidarité FTQ and Merck & Co. This fund is the latest addition to the GeneChem portfolio of funds, a fund manager in existence since 1997. AmorChem’s innovative business model involves financing research-stage projects to enable them to reach pre-clinical proof-of-concept (“POC”) in a semi-virtual mode within 18-24 months. The fund seeks to generate returns through a two-pronged exit strategy: sell projects having reached POC to large biotechnology or pharmaceutical companies; or bundle them into new spin-out companies. AmorChem using external resources will manage the projects. To that end, AmorChem has established a strategic partnership with the Biotechnology Research Institute in order to

access its R&D platforms. In addition to enabling projects requiring small molecules as tools or drug leads, AmorChem has founded NuChem Therapeutics, Inc., a medicinal chemistry contract-research company. Follow AmorChem on Twitter @amorchem

### **About NuChem**

NuChem Therapeutics, Inc., ([www.nuchemtherapeutics.com](http://www.nuchemtherapeutics.com)) is a medicinal chemistry contract-research company wholly-owned by AmorChem. With laboratories situated at the Biotechnology Research Institute in Montreal, the company is led by Dr. Daniel Guay, formerly of Merck Canada and it offers full service chemistry support to early-stage projects.

### **About Roche external drug discovery**

The external drug discovery group is part of the Roche Pharma Research and Early Development (pRED) approach to accessing external innovation. The group consists of dedicated team members with deep experience and expertise to support external collaborators' rapid development of clinical candidates. Roche Partnering works with this pRED group to find potential partners and lead the business development to support pRED's external innovation efforts. In a typical external drug discovery partnership Roche provides shared R&D funding and, as needed, in-kind contribution, while external scientists and entrepreneurs independently drive discovery and preclinical development.

### **About Roche**

Headquartered in Basel, Switzerland, Roche is a leader in research-focused healthcare with combined strengths in pharmaceuticals and diagnostics. Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and neuroscience. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management. Roche's personalised healthcare strategy aims at providing medicines and diagnostics that enable tangible improvements in the health, quality of life and survival of patients. Founded in 1896, Roche has been making important contributions to global health for more than a century. Twenty-four medicines developed by Roche are included in the World Health Organisation Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and chemotherapy.

In 2013 the Roche Group employed over 85,000 people worldwide, invested 8.7 billion Swiss francs in R&D and posted sales of 46.8 billion Swiss francs. Genentech, in the United States, is a wholly owned

member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit [www.roche.com](http://www.roche.com).

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