



## Sanofi and MyoKardia Announce Groundbreaking Collaboration to Develop Targeted Therapies for Patients with Genetic Heart Disease

*- Up to \$200 Million Collaboration to Support Pioneering Science and New Treatments -*

**Paris and South San Francisco, Calif. - September 17, 2014** - Sanofi (EURONEXT: SAN and NYSE: SNY) and [MyoKardia, Inc.](#), a privately-held company leading the development of precision therapies for genetic heart disease, announced today a worldwide collaboration to discover and develop first-of-its-kind targeted therapeutics for heritable heart diseases known as cardiomyopathies, the most common forms of heart muscle disease. The collaboration builds upon MyoKardia's pioneering science, which hopes to correct the disruptive effects that disease mutations have on heart muscle function.

The collaboration, representing one of the largest research and development commitments to genetic forms of cardiomyopathy, encompasses three MyoKardia programs. Two of these programs are focused on hypertrophic cardiomyopathy (HCM) and the other is focused on dilated cardiomyopathy (DCM). The collaboration provides up to \$200 million in equity investments, milestone payments and research and development services through 2018, of which \$45 million has already been received in an upfront licensing fee and an initial equity investment. In addition, Sanofi and MyoKardia will equally share development costs on the HCM programs following initial demonstration of efficacy in patients, with Sanofi fully covering the development costs of the DCM program.

The collaboration is an outgrowth of Sanofi's Sunrise initiative, a strategic partnership model that seeks to invest in early stage opportunities that align with Sanofi's expert development and commercialization abilities. The commitment of resources from Sanofi will accelerate and broaden MyoKardia's basic disease research and will support the shared commitment of bringing desperately needed therapies to patients.

*"This collaboration illustrates Sanofi's research and development philosophy for Sunrise projects. It combines in a meaningful way the unique expertise in rare and cardiovascular diseases of our top scientists with that of the best innovators in the world, like MyoKardia's founders and scientists, to achieve real breakthroughs in medicine,"* said Dr. Elias Zerhouni, President Global R&D for Sanofi.

*"MyoKardia's research represents the first hope for targeted treatments that address the primary cause of each patient's disease,"* said Tassos Gianakakos, Chief Executive Officer, MyoKardia. *"By genetically defining HCM and DCM into several underlying rare genetic diseases, MyoKardia's candidate therapies have the potential to be developed far more efficiently than traditional cardiovascular drugs. The collaboration extends MyoKardia's competitive advantage, allowing advancement of a broad portfolio of important treatments for patients."*

Within the collaboration, MyoKardia will drive research and worldwide development activities through early human efficacy studies. Thereafter, MyoKardia will lead worldwide development and U.S. commercial activities for the two HCM programs, where it has retained product rights, and Sanofi will lead global development and commercial activities for DCM where it has obtained worldwide rights, and ex-U.S. regulatory and commercial activities to the two HCM programs where it has ex-U.S. commercialization rights. Sanofi also has the option to co-promote in the U.S. for potential expanded cardiovascular diseases outside of the genetically targeted indications for either



of the HCM programs, with MyoKardia having the option to co-promote the DCM program in the U.S.

*“We are very excited to embark on this deep collaboration with MyoKardia to identify first-in-class, life-changing therapies for HCM and DCM patients,”* said Dr. Kathy Bowdish, Vice President Global R&D and Head of Sunrise. *“This opportunity allows us to advance our collective knowledge and bench strength in this exciting and emerging field. This creative collaboration truly demonstrates Sanofi’s open innovation model.”*

### **About Genetic Cardiomyopathies**

HCM and DCM are types of heritable heart diseases that are caused by mutations in the genes of the proteins that are primarily responsible for the contraction of the heart muscle. HCM is the leading cause of sudden cardiac death in young adults, and DCM, the leading genetic illness requiring heart transplantation, together are believed to affect nearly 800,000 children and adults in the U.S. alone. Available treatments for patients suffering from these forms of heart disease have been approved for other illnesses and only treat the symptoms of their disease. As their disease progresses, it can require invasive procedures including heart transplantation, and can lead to sudden cardiac death and complications associated with heart failure, including stroke.

HCM is believed to be the most common heritable cardiovascular disease, thought to affect one in 500 people in the general population. HCM is characterized by an abnormal thickening and stiffening of the walls of the heart’s main pumping chamber, the left ventricle. Disease-causing HCM mutations make it difficult for the heart to relax and refill with fresh blood between beats, causing shortness of breath and sometimes dizziness, fainting or chest pain. About one-fourth of HCM patients experience an abnormal heart rhythm, called atrial fibrillation, which profoundly increases the risk of potentially debilitating or even fatal stroke.

Genetically-defined DCM, estimated to occur in one in 2,500 people in the general population, is a condition in which the heart becomes enlarged and weakened, and is unable to pump enough blood to the rest of the body. DCM is also known to be associated with harmful blood clots, which can cause strokes, and with irregular and harmful heartbeats and sudden cardiac death. More than 20 percent of heart transplantations are performed every year for patients with genetic DCM.

### **About Sanofi**

Sanofi, an integrated global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients’ needs. Sanofi has core strengths in the field of healthcare with seven growth platforms: diabetes solutions, human vaccines, innovative drugs, and consumer healthcare, emerging markets, animal health and the new Genzyme. Sanofi is listed in Paris (EURONEXT: SAN) and in New York (NYSE: SNY).

To learn more about Sunrise, please visit [www.sanofi-sunrise.com](http://www.sanofi-sunrise.com).

### **About MyoKardia**

MyoKardia, Inc. is pioneering a precision medicine approach to treat patients with genetic heart disease. By combining leading-edge cardiovascular genetics with recent advances in heart muscle biochemistry, MyoKardia seeks to make a meaningful difference in the lives of people suffering from these diseases. Leveraging powerful genetic insights MyoKardia intends to classify these heritable cardiovascular diseases into a series of underlying rare genetic disorders, enabling physicians to treat patients based on their unique genetic and clinical profiles. MyoKardia has helped organize an international patient registry, the [Sarcomeric Human Cardiomyopathy Registry \(SHaRe\)](#) to help in this effort. As a result, MyoKardia’s therapeutic candidates have the potential to be developed far more efficiently than traditional cardiovascular drugs – matching the appropriate therapy to each patient. Launched in 2012, MyoKardia was founded by world-class experts in cardiovascular disease, cardiac muscle biology and genetics, and is funded by leading healthcare investor Third Rock Ventures. For more information, please visit [www.myokardia.com](http://www.myokardia.com).



### **Sanofi Forward-Looking Statements**

*This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. Forward-looking statements are generally identified by the words “expects”, “anticipates”, “believes”, “intends”, “estimates”, “plans” and similar expressions. Although Sanofi’s management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, the Group’s ability to benefit from external growth opportunities, trends in exchange rates and prevailing interest rates, the impact of cost containment policies and subsequent changes thereto, the average number of shares outstanding as well as those discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under “Risk Factors” and “Cautionary Statement Regarding Forward-Looking Statements” in Sanofi’s annual report on Form 20-F for the year ended December 31, 2013. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.*

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### **Contacts:**

#### **Sanofi**

##### **Media Relations**

Laurence Bollack

Tel: +33 (0) 1 53 77 46 46

E-mail: [mr@sanofi.com](mailto:mr@sanofi.com)

##### **Investor Relations**

Sebastien Martel

Tel: +33 (0)1 53 77 45 45

E-mail: [IR@sanofi.com](mailto:IR@sanofi.com)

##### **Global R&D Communications**

Amy Ba, Ph.D.

Tel: +1 617-665-4851

E-mail: [amy.ba@sanofi.com](mailto:amy.ba@sanofi.com)

#### **MyoKardia**

##### **Sheryl Seapy**

Pure Communications

[sheryl@purecommunicationsinc.com](mailto:sheryl@purecommunicationsinc.com)

Tel: 949-903-4750