



FOR IMMEDIATE RELEASE

Yissum Introduces a Novel Technology for Drug Screening and Testing of Orphan Diseases without Utilizing Animal Models

Novel technology utilizes stem cells as models for genetic disorders and enables large scale screening of small molecule drugs

Jerusalem, Israel, July 27, 2015 – Animal models are commonly used to study novel molecules for various diseases, but this approach suffers from various limitations. For example, numerous developmental diseases cannot be studied using animal models, nor by isolating cells from patients.

Now Yissum, the Research and Development Company of the Hebrew University introduces a novel platform technology for screening and evaluating small molecules as potential drugs for various human genetic disorders which are essentially orphan diseases. The method, developed by Prof. Nissim Benvenisty, Director, The Azrieli Center for Stem Cells and Genetic Research at the Hebrew University, utilizes human pluripotent stem cells (hPSCs) as a model system that overcomes several limitations inherent to animal models.

As part of the novel approach, Prof. Benvenisty's Center has generated hPSCs representing human genetic disorders, such as Fragile X syndrome or Down syndrome, as well as other genetic conditions such as some types of cancers or hereditary diabetes. Prof. Benvenisty and his group have developed 15 disease cell models for 13 genetic disorders, which can serve not only to understand the molecular basis underlying these diseases, but also as platforms for screening small molecules to treat these disorders. In addition, since the cellular models are derived from human cells, they can also serve to validate potential drugs intended for the treatment of human developmental genetic disorders.

Yaccov Michlin, CEO of Yissum, commented, “The new disease model system developed by Prof. Benvenisty offers a complementary approach that can address many of the hurdles inherent to animal models. We are certain that this novel method will have a significant impact on drug screening and development, and Yissum is currently looking for partners in order to further advance this promising approach.”

Animal models are an important step in the research and development of many drugs, but often fall short due to various limitations. This cellular system, based on human stem cells, enables, for example, cost-effective high throughput screening of small molecules as potential drugs for a variety of genetic and other conditions, screening which is not possible using many animal models. Furthermore, the ability to manipulate the genome of these cells in order to emulate various genetic diseases offers an extensive potential to study, understand and develop new therapies for many complex diseases with an underlying genetic basis.

About Yissum

Yissum Research Development Company of the Hebrew University of Jerusalem Ltd. was founded in 1964 to protect and commercialize the Hebrew University's intellectual property. Products based on Hebrew University technologies that have been commercialized by Yissum currently generate \$2 Billion in annual sales. Ranked among the top technology transfer companies in the world, Yissum has registered over 8,960 patents covering 2,500 inventions; has licensed out 785 technologies and has spun out 96 companies. Yissum's business partners span the globe and include companies such as Novartis, Microsoft, Johnson & Johnson, Merck, Intel, Teva, ICL and many more. For further information please visit www.yissum.co.il

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