

#### Sphingolipid metabolism modifiers (Yissum) code: 12-2006-158 Shimon Gatt, HUJI, Faculty of Medicine, Cellular biochemistry and human genetics Arie Dagan, HUJI, Faculty of Medicine, Biochemistry

## Compounds for treating cancer, malaria and lipid storage diseases

Categories	Oncology, antimalarial, Niemann-Pick, Gaucher
Development Stage	A large number of analogs have been synthesized and screened for anti cancer activity in various tissue culture cell lines. Three analogs were selected and showed activity when tested in mice models of Human Prostate, Melanoma and Pancreatic tumors.
Patent Status	U.S granted (No. 6,756,504), Europe granted (No. 1430019), Filed in Japan
Market Size	The total worldwide estimated market for prostate, melanoma and pancreatic cancers is more than \$20B. Gaucher disease is more than \$840 million

## Highlights

- Novel new compounds
- Anticancer activity observed with subcutaneous and in metastatic cancer mice models of prostate, pancreas and melanoma. Analogs were injected intradermally or intraperitoneally, or given per os to mice bearing human tumors.
- Provides powerful stand alone or adjunctive therapy with irradiation or chemotherapy for cancer
- Compounds for treating cancer, parasitic diseases such as malaria, lipid storage diseases such as Gaucher, Niemann-Pick, Tay-Sachs and more
- Elevates cellular ceramide, inducing cytotoxicity and death by apoptosis

# **Our Innovation**

Novel synthetic compounds - non-natural analogs of sphingolipids – that modify sphingolipid metabolism

## **Key Features**

- Induce apoptosis and death to cancer cells.
- Reduce human tumors (eg, prostate, melanoma, pancreas) in nude mice.
- Reduce biosynthesis of sphingomyelin and glycolipids in lipid-storage diseases. Also inhibit sphingolipid hydrolases and can be used for "chaperone" therapy of these diseases.
- Kill malaria parasites in nanomolar concentrations.

## **Development Milestones**

- Further in vivo mouse models
- Pharmacokinetic pharmacodynamic experiments
- In vivo human testing



## **The Opportunity**

- Provides more efficacious cancer treatment
- Addresses needs for expanding cancer market
- Legislative and economic incentives have created a positive climate for developing drugs and treatments for rare diseases; many orphan drugs are sold at high prices that can reach several hundred thousand US dollars per year; new orphan drugs now provide help for more than 11 million people in the USA alone
- The global market for antimalarial drugs of the order of \$100-120 million per year

#### **Contact for more information:**

Ariela Markel 🖂, VP, Business Development, Healthcare, +972-2-6586608

Yissum Research Development Company of the Hebrew University of Jerusalem Hi-Tech Park, Edmond J. Safra Campus, Givat-Ram, Jerusalem P.O. Box 39135, Jerusalem 91390 Israel Telephone: 972-2-658-6688, Fax: 972-2-658-6689