

## **Pfizer and Protalix to Develop and Commercialize Treatment for Gaucher's Disease**

### **Target Health is the CRO Partner**

Target Health congratulates its client Protalix for this excellent opportunity.

Target Health was Protalix's CRO partner and together with Cato Israel, who monitored outside of North America, we took this product from toxicology to the pre-IND meeting, IND submission, Phase I, Phase III, Orphan Drug, Fast Track, Expanded Access, multiple FDA meetings and now the eCTD NDA submission. Target e\*CRF® and Target Document® were used for the pivotal trial and is now being used in four additional protocols. This development program was the best case of a Sponsor/CRO/FDA partnership. The pre-IND meeting took place in June 2004, and last patient last visit was September 2009.

Target Health's team was lead by Glen Park PharmD. Dr. Park joined Target Health in 2005 and since then has been directly involved with approvals of 1 NDA (head lice), 1 MAA (emergency contraception) and 1 PMA (adhesion prevention in the newborn). There are currently 2 NDA submissions in review (emergency contraception and cystic fibrosis).

### **Press Release**

Pfizer Inc. and Protalix, Ltd. today announced that they have entered into an agreement to develop and commercialize taliglucerase alfa, a plant-cell expressed form of glucocerebrosidase (GCD) in development for the potential treatment of Gaucher's disease. Under the terms of the agreement, Pfizer will receive exclusive worldwide licensing rights for the commercialization of taliglucerase alfa, while Protalix will retain the exclusive commercialization rights in Israel. Taliglucerase alfa is the first enzyme replacement therapy derived from a proprietary plant cell-based expression platform using genetically engineered carrot cells.

With the successful completion of Phase III clinical studies, Protalix is preparing to complete a rolling NDA with the FDA. The FDA has granted Orphan Drug designation and Fast Track status, facilitating the development and expediting the review of drugs to treat rare conditions or diseases, as well as an Emergency Use Authorization. The FDA has also requested, and subsequently approved, an Expanded Access Program (EAP) treatment protocol. Taliglucerase alfa is currently being provided to Gaucher's patients in the U.S. under the EAP protocol, as well as to patients in the European Union under a compassionate use protocol.

Peter L. Saltonstall, President and CEO, National Organization for Rare Disorders (NORD) stated, "NORD is always pleased when treatment options are expanded for people with rare diseases. We welcome Pfizer's commitment to the rare disease arena, and look forward to working with both Pfizer and Protalix in support of increased options for patients and families affected by rare diseases."