# Protalix BioTherapeutics' Taliglucerase Alfa Phase III Results Published in Blood, the Journal of the American Society of Hematology

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Protalix BioTherapeutics, Inc. (NYSE-AMEX: PLX, TASE: PLX), today announced that an article entitled "Pivotal Trial with Plant-Cell-Expressed Recombinant Glucocerebrosidase, taliglucerase alfa, a Novel Enzyme Replacement Therapy for Gaucher Disease" has been published in *Blood*, the Journal of the American Society of Hematology. The full article can be found on the American Society of Hematology's website at http://bloodjournal.hematologylibrary.org. (First Edition Paper, pre-published online September 6, 2011; DOI 10.1182/blood-2011-07-366955)

The reported study is based on the Company's phase III clinical trial of taliglucerase alfa for the treatment of Gaucher disease. The authors, who include among others specialists in the treatment of Gaucher disease that served as investigators in our phase III clinical trial, concluded that treatment of 31 naive Gaucher disease patients with taliglucerase alfa at doses of 60 U/kg or 30 U/kg administered intravenously every two weeks for nine months reduced mean spleen volume by 38.0 percent and 26.9 percent, respectively. Additionally, patients treated with taliglucerase alfa in the phase III clinical trial demonstrated an increase in hemoglobin, decrease in liver volume and increase in platelet count.

"The sustained efficacy and safety that we observed among Gaucher patients receiving taliglucerase alfa in this study suggests that it is a valuable long-term treatment option for patients with Gaucher disease pending regulatory approval," stated Professor Ari Zimran, M.D., Director of the Gaucher Clinic in Shaare Zedek Medical Center, Jerusalem, Israel and lead clinical investigator.

**Phase III Trial Design**

The pivotal Phase III clinical trial was a world-wide, multi-center, randomized, double-blind, parallel group, dose-ranging study to assess the safety and efficacy of taliglucerase alfa in 31 treatment-naive patients suffering from Gaucher disease. In the trial, patients were selected randomly for one of two dosing arms (60 U/kg or 30 U/kg) and received intravenous infusions of taliglucerase alfa once every two weeks for a nine month period. The primary endpoint of the study was a mean reduction from baseline in spleen volume after nine months, as measured by MRI. Major secondary endpoints were an increase in hemoglobin, decrease in liver volume and increase in platelet count. The trial was performed in 11 centers throughout Europe, Israel, North America, South America and South Africa.

**Phase III Trial Results**

Taliglucerase alfa significantly reduced mean spleen volume after nine months compared with baseline in both treatment groups. The 60 U/kg group demonstrated a statistically significant mean reduction in spleen volume of 38.0 percent ($p<0.0001$) and the 30 U/kg group demonstrated a statistically significant mean reduction in spleen volume of 26.9 percent ($p<0.0001$). In addition, the primary endpoint was achieved in both treatment groups after only six months of therapy.

Statistically significant improvements were also observed for the secondary endpoints after nine months when compared to baseline for the 60 U/kg dose. Patients demonstrated a mean increase in hemoglobin of 2.2 g/dL or 22.2 percent ($p<0.0001$), a mean decrease in liver volume of 11.1 percent ($p<0.0001$) and a mean elevation in platelet count of 41,494 ml or 72.1 percent ($p=0.0031$). For patients in the 30 U/kg dose, statistically significant improvements after nine months compared with baselines were observed for hemoglobin level (increased 1.6 g/dL or 14.8 percent; $p=0.0010$) and liver size (decreased 10.48 percent; $p=0.0041$); a nominal elevation in platelet count was also seen (11,427 ml or 13.7 percent; $p=0.0460$).

The safety analysis for both treatment groups showed that taliglucerase alfa was well tolerated and no serious or severe adverse events were reported. Two patients in the trial developed antibodies to taliglucerase alfa and no patients developed neutralizing antibodies. In addition, two patients experienced hypersensitivity reactions to taliglucerase alfa. No anti-taliglucerase antibodies were detected in these patients and both reactions were successfully treated in the physicians' office.

On November 30, 2009, Pfizer and Protalix BioTherapeutics, Inc. entered into an agreement to develop and commercialize taliglucerase alfa. Pfizer remains fully dedicated to the Gaucher disease community and to its relationship with Protalix and has worked closely with Protalix providing technical, analytical and regulatory expertise.

**About Protalix**

Protalix is a biopharmaceutical company focused on the development and commercialization of recombinant therapeutic proteins expressed through its proprietary plant cell based expression system, ProCellEx(TM). Protalix's unique expression system presents a proprietary method for developing recombinant proteins in a cost-effective, industrial-scale manner in an environment free of mammalian components and viruses. Protalix's lead compound, taliglucerase alfa, an enzyme replacement therapy for the treatment of Gaucher disease, completed phase III development. To date, marketing applications have been submitted for taliglucerase alfa in the United States, the European Union, Brazil, Israel and Australia. Protalix's development pipeline also includes the following product candidates: PRX-102, a modified version of the recombinant human alpha-GAL-A protein for the treatment of Fabry disease; PRX-105, a pegylated recombinant human acetylcholinesterase in development for several therapeutic and prophylactic indications, a biodefense program and an organophosphate-based pesticide treatment program; an orally-delivered glucocerebrosidase enzyme that is naturally encased in carrot cells, also for the treatment of Gaucher disease; pr-antiTNF, a similar plant cell version of etanercept (Enbrel(TM)) for the treatment of certain immune diseases such as rheumatoid arthritis, juvenile idiopathic arthritis, ankylosing spondylitis, psoriatic arthritis and plaque psoriasis; and others.

**Forward Looking Statements**

To the extent that statements in this press release are not strictly historical, all such statements are forward-looking, and are made pursuant to the safe-harbor provisions of the Private Securities Litigation Reform Act of 1995. The terms "anticipate," "believe," "estimate," "expect" and "intend" and other words or phrases of similar import are intended to identify forward-looking statements. These forward-looking statements are subject to known and unknown risks and uncertainties that may cause actual future experience and results to differ materially from the statements made. These

statements are based on our current beliefs and expectations as to such future outcomes. Drug discovery and development involve a high degree of risk. Factors that might cause material differences include, among others: risks relating to the review process of the FDA, the European Medicines Agency (EMA), other foreign regulatory bodies and other governmental regulatory bodies, including the risk that regulatory authorities may find that the data from our clinical trials and other studies is insufficient for regulatory approval; risks relating to delays in the FDA's, the EMA's or other foreign regulatory authorities' approval of any applications we file or refusals to approve such filings, including the NDA we filed with the FDA for taliglucerase alfa for the treatment of Gaucher disease; applicable regulatory authorities may refuse to approve the marketing and sale of a drug product even after acceptance of an application we file for the drug product; risks relating to the completion of our clinical trials; and other factors described in our filings with the Securities and Exchange Commission. Companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in advanced or late-stage clinical trials, even after obtaining promising earlier trial results or in preliminary findings for such clinical trials. Further, even if favorable testing data is generated from clinical trials of drug products, the FDA, EMA or any other foreign regulatory authority may not accept or approve an NDA filed by a pharmaceutical or biotechnology company for such drug product. Failure to obtain approval from the FDA, EMA or any other foreign regulatory authority of any of our drug candidates in a timely manner, if at all, will severely undermine our business and results of operations by reducing our potential marketable products and our ability to generate corresponding product revenues. The statements in this release are valid only as of the date hereof and we disclaim any obligation to update this information.

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