

PROTALIX BIOTHERAPEUTICS

CORPORATE PRESENTATION
FEBRUARY 2021



PROTALIX
Biotherapeutics

Note Regarding Forward-Looking Statements

This presentation (the “Presentation”) contains forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934, as amended. The forward-looking statements, including, among others, statements regarding expectations as to regulatory approvals, market opportunity for, and potential sales of, the Company’s product and product candidates, goals as to product candidate development and timing of the Company’s clinical trials, are based on the Company’s current intent, belief and expectations. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that might cause material differences include, among others: failure or delay in the commencement or completion of the Company’s preclinical and clinical trials which may be caused by several factors, including: that the FDA might not grant marketing approval for PRX 102 by the PDUFA date or at all and, if approved, whether PRX 102 will may have significant limitations on its use or be commercially successful; risk that the FDA will request additional data or other conditions of the Biologics License Application (BLA) filing for Accelerated Approval of PRX 102; failure or delay in the commencement or completion of our preclinical and clinical trials which may be caused by several factors, including: slower than expected rates of patient recruitment; unforeseen safety issues; determination of dosing issues; lack of effectiveness during clinical trials; inability to monitor patients adequately during or after treatment; and inability or unwillingness of medical investigators and institutional review boards to follow our clinical protocols; risks associated with the novel coronavirus (COVID-19) outbreak, which may adversely affect our business, preclinical studies and clinical trials; the risk that the results of the clinical trials of the Company’s product candidates will not support the Company’s claims of safety or efficacy, that the Company’s product candidates will not have the desired effects or will be associated with undesirable side effects or other unexpected characteristics; risks related to the Company’s ability to finance future research and development activities, general and administrative expenses and working capital; risks relating to the Company’s ability to maintain and manage the Company’s relationship with Chiesi Farmaceutici S.p.A. and any other collaborator, distributor or partner; risks related to the Company’s commercialization efforts for alfataglicerase in Brazil; risks relating to the compliance by Fundação Oswaldo Cruz with its purchase obligations and related milestones under the supply and technology transfer agreement; the risk that despite the FDA’s grant of fast track designation for PRX-102, the Company may not experience a faster development process, review or approval compared to applications considered for approval under conventional FDA procedures; risks related to the FDA’s ability to withdraw the fast track designation at any time; the Company’s dependence on performance by third party providers of services and supplies, including without limitation, clinical trial services; delays in the Company’s preparation and filing of applications for regulatory approval; the inherent risks and uncertainties in developing drug platforms and products of the type the Company is developing; the impact of development of competing therapies and/or technologies by other companies and institutions; potential product liability risks, and risks of securing adequate levels of product liability and other necessary insurance coverage; and other factors described in the Company’s filings with the U.S. Securities and Exchange Commission. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of today’s date. The Company undertakes no obligation to update or revise the information contained in this Presentation whether as a result of new information, future events or circumstances or otherwise.



Investment Highlights

Plant cell expressed recombinant proteins with improved therapeutic profiles



Revenue Generating

FDA approved, commercially marketed drug for Gaucher disease. Eleyso and alfataliglicerase.



Fabry Disease

FDA accepted BLA in August 2020 and granted Priority Review for lead asset pegunigalsidase alfa for the treatment of adult patients with Fabry disease. The company is conducting three ph:3 trials. Interim results from the ongoing BALANCE trial is expected in 1H21; for EMA submission.



Partnerships

Chiesi, Pfizer and Fundacao Oswaldo Cruz (Fiocruz)



Platform

Proprietary ProCellEx[®] platform for recombinant protein expression cGMP manufacturing facility successfully inspected and audited by multiple regulatory agencies, including the FDA & EMA.



Pipeline

Uricase (PRX-115) for the treatment of Refractory Gout, Dnase I (PRX-119) for multiple indications, as well as other products in seed and preclinical phases.



Finances

Well capitalized with potential significant regulatory & commercial milestone payments (\$1B) & robust royalty opportunities (15-40%). Executed \$30M At-the-Market Facility led by Bank of America (BoFA) in Q3'20.

Note: cGMP = Current Good Manufacturing Practice.

ProCellEx® Platform

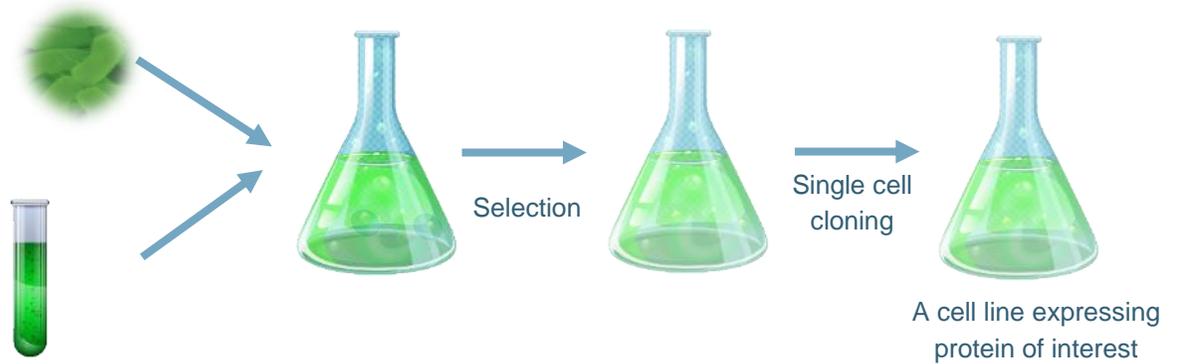
First and only company to gain FDA approval of a protein produced through plant cell-based expression system

Unique capabilities of tailoring genetic engineering and protein engineering tools for pre/post-production modifications, customized for each individual protein candidate

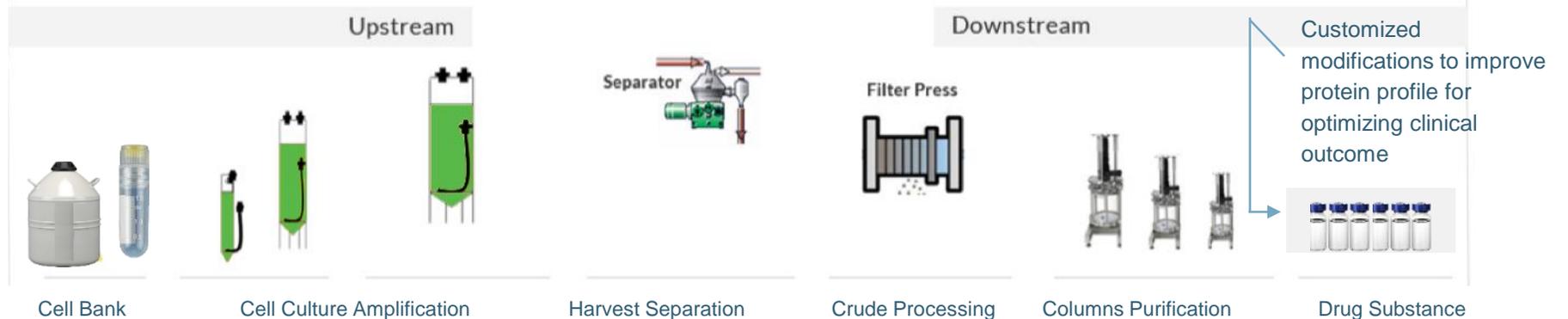
DEVELOPMENT OF TRANSGENIC CELL LINES FOR PRODUCTION OF TARGET PROTEIN

Agrobacterium mediated cell transformation using the natural capability of the agrobacteria to transfer DNA fragment into a plant chromosome

Proprietary Plant cell lines



PROCESS OVERVIEW



Advantages of Proprietary Plant Cell-Based Platform (ProCellEx®)

> **Biologic Optimization**

Experienced internal interdisciplinary capabilities to improve biologic dynamics (e.g., glycosylation profile, half-life, immunogenicity) of a protein

> **Intellectual Property**

IP advantages due to proprietary manufacturing processes and development of 2nd generation, improved products

> **Simplified Production Process**

Production at room temperature, simplified maintenance with no risk of viral contamination from mammalian components



> **Can Handle Complexity**

Ability to express certain proteins that are difficult to express in other systems

> **Delivery**

Protein protected inside plant cell enabling oral delivery of proteins via gastrointestinal tract

> **Flexible Scale-Up**

Flexible infrastructure design allows for rapid horizontal scale-up (or scale-down), as required

Source: Company Information.

Elelyso[®] for Gaucher Disease

First plant cell derived recombinant protein approved by the FDA

Validation of the ProCellEx[®] platform

Gaucher disease is a rare genetic disorder characterized by the deposition of glucocerebroside in cells of the macrophage-monocyte system. Possible symptoms include enlarged liver and spleen, various bone disorders, easy bruising, and anemia. Left untreated, Gaucher disease can cause permanent body damage and decrease life expectancy.

Elelyso is approved in 24 markets¹. Monetized through a world-wide exclusive license agreement with Pfizer in 2009, amended in 2015 (excluding Brazil).



Elelyso provides a consistent (and growing) revenue stream for Protalix while validating the **ProCellEx** platform technology and demonstrating the company's manufacturing and production expertise and ability to bring a treatment from concept to market production.



Sales of ~\$9.1M in Brazil (2019)



~27% market share in Brazil



10-15% annual growth expected over next 3-5 years



1. Approved in 23 markets including the US, Australia, Canada, Israel, Brazil, Russia and Turkey. The European Committee for Medicinal Products for Human Use (CHMP) gave a positive opinion but also concluded that the medicine cannot be granted marketing authorization in the EU because of the ten-year market exclusivity that had been granted to Vpriv[®] (Shire), which was authorized in August 2010 for the same condition.

Product Pipeline

Recombinant proteins with improved therapeutic profiles that target unmet medical needs and established pharmaceutical markets

| | Discovery and Preclinical | Phase 1 | Phase 2 | Phase 3 | Marketing Application |
|---------------------------------|---------------------------------|---------|-----------------|---------|-----------------------|
| pegunigalsidase alfa (PRX-102) | Fabry Disease | | | | |
| tulinercept (OPRX-106) | Inflammatory Bowel Disease (UC) | | | | |
| alidornase alfa (PRX-110) | Various | | Cystic Fibrosis | | |
| uricase (PRX-115) | Gout | | | | |
| Long Acting A DNase I (PRX-119) | NETs Related Diseases | | | | |

All of our pipeline candidates are recombinant proteins expressed via our proprietary ProCellEx® system.



Fabry Disease

Rare Genetic Disease
occurs in one of every
40,000 people.

Growing Market
~\$2B+ growing market
(expected CAGR ~10%)

**Fabry disease is primarily
treated with**
enzyme replacement therapy
(ERT) to replace the missing
alpha-Galactosidase-A enzyme
with a recombinant form of the
protein via intravenous infusion
1x every 2 weeks.

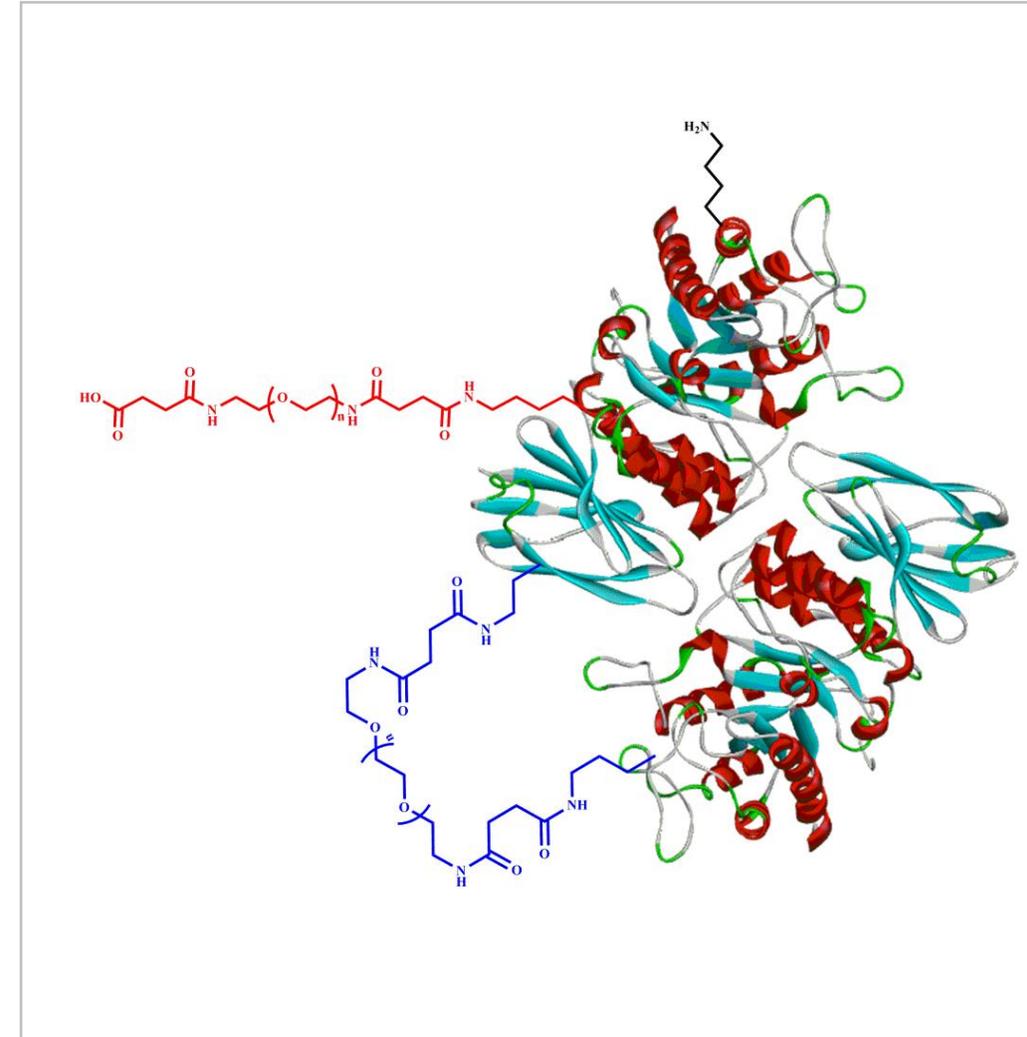
Fabry Disease Competitive Landscape

| Product Name | Fabrazyme® | Replagal® | Galafold® |
|----------------|--|--|--|
| Parent Company | Sanofi | Shire | Amicus |
| Mechanism | ERT | ERT | Pharmacological chaperone |
| Indication | <p>Fabrazyme is indicated for use in patients with Fabry disease. Fabrazyme reduces GL-3 deposition in capillary endothelium of the kidney and certain other cell types. (U.S.)</p> <p>Fabrazyme is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency). Fabrazyme is indicated in adults, children and adolescents aged 8 years and older. (E.U.)</p> | <p>Replagal is indicated for long-term enzyme replacement therapy in patients with a confirmed diagnosis of Fabry Disease (α-galactosidase A deficiency). (E.U.)</p> | <p>GALAFOLD is an alpha-galactosidase A (alpha-Gal A) pharmacological chaperone indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data.</p> <p>This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell KIC GL-3 substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. (U.S.)</p> <p>Galafold is indicated for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation (E.U.)</p> |
| Approval Date | 2003 (U.S.); 2001 (E.U.) | Not approved in US ; 2001 (E.U.) | 2018 (U.S.); 2016 (E.U.) |
| Treatment Type | Bi-weekly infusions | Bi-weekly infusions | Oral |
| Dosing | 1mg/kg every 2 weeks | 0.2mg/kg every 2 weeks | 123 mg every other day |



Pegunigalsidase Alfa is Designed For (MOA):

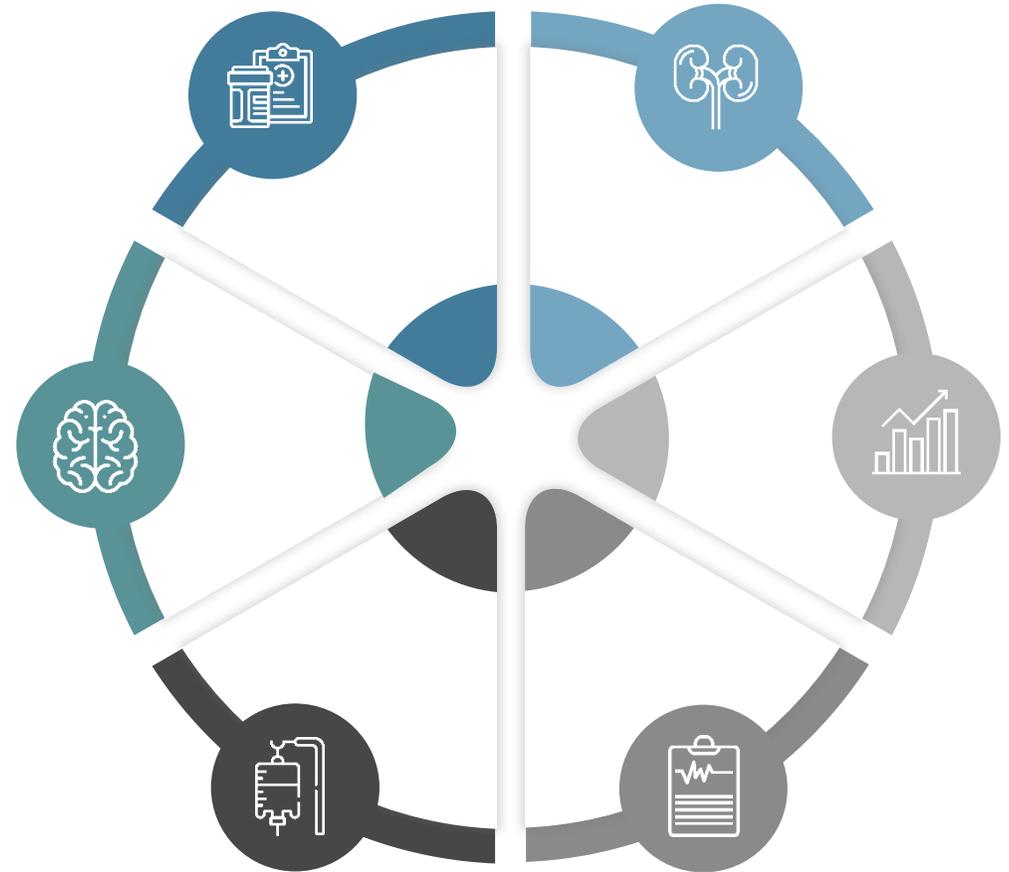
- Pegunigalsidase is a PEGylated enzyme designed to be potentially superior to the currently approved ERTs
- Covalent linked via short 2Kd PEG, stable enzyme with extended circulatory and tissue half-life and enzyme
- Continuous coverage/presence of enzyme over infusion intervals without compromising the enzyme activity and internalization to target organ and cells
- Providing potentially increased enzyme exposure and enhanced activity to target organs and sustained hydrolysis and prevention of accumulation and re-accumulation of substrate
- PEGylation potentially reduces immunogenicity by masking immunogenic epitopes together with the continued presence has the potential to induce immune tolerance
- PEGylation potentially reduces the cross reactivity and reduces serum mediated enzyme inhibition of already existing antibodies (in patients previously treated with other ERT)
- Two optional dosing regimens based on unique product characteristics



Potential Advantages of pegunigalsidase alfa (PRX-102)

PEGUNIGALSIDASE ALFA

- Delivery of active enzyme into the target organs .
- Reduction of globotriaosylceramide (Gb₃) burden in kidney biopsies which demonstrates potential efficacy based on proven Mechanism of Action.
- Clinical improvement measured by attenuation of disease progression.
- Improved QOL: less IV burden & fewer infusions per month/year.
- Reduced immunogenic response due to PEGylation and enhanced pharmacokinetic properties of pegunigalsidase alfa.
- Multiple dosing/regimen options are favorable to both patients and physicians.

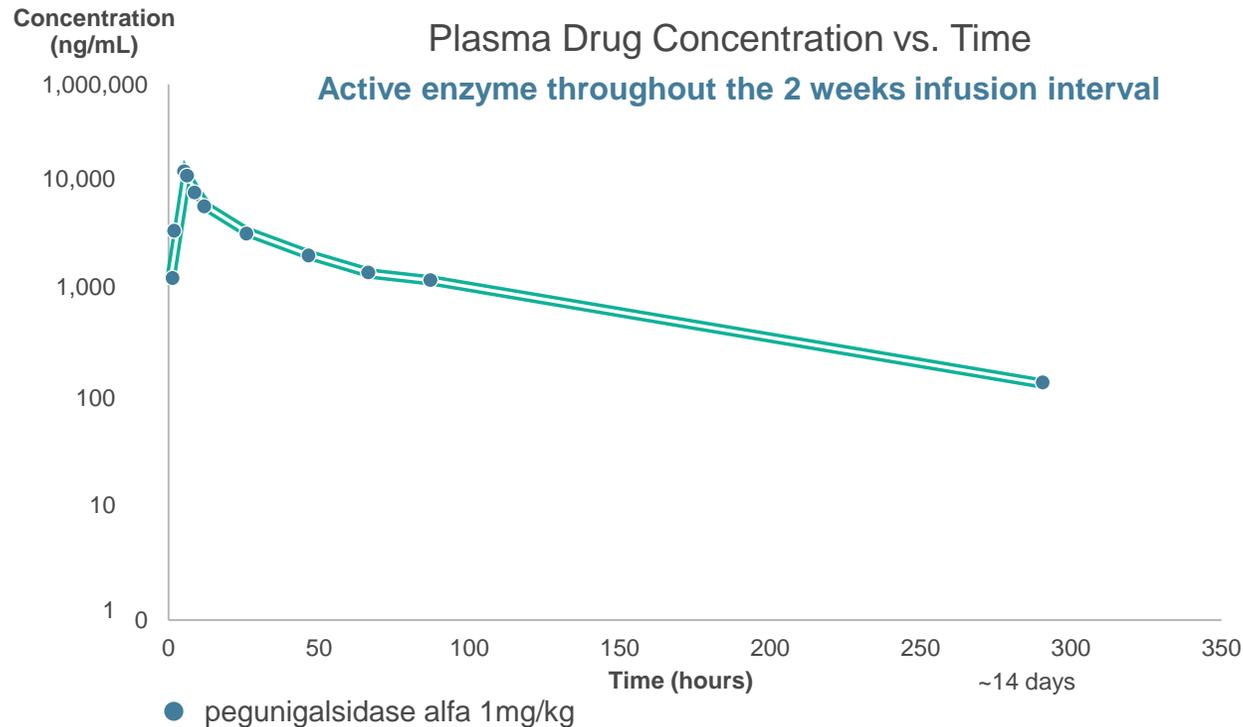


Phase I/II Study - Development Rationale

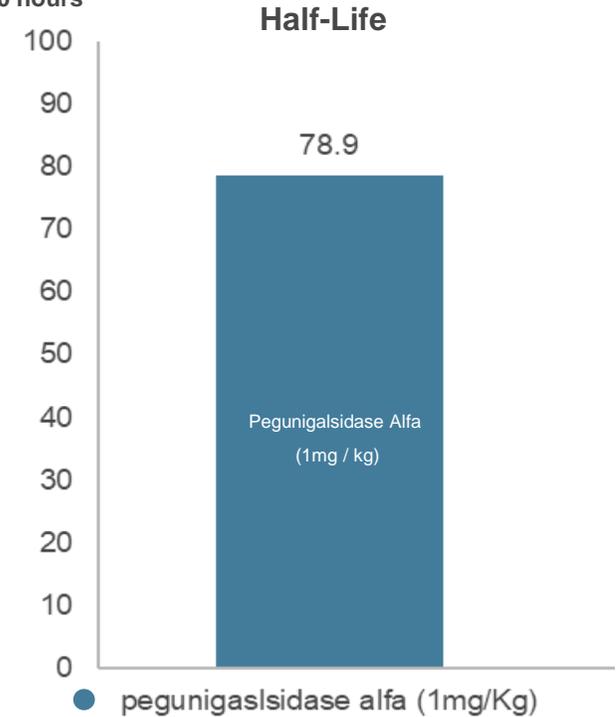
High levels of active available enzyme → potentially improved clinical benefit

- Chemically modified plant cell derived PEGylated covalently bound homodimer
- In Phase I/II clinical trial, pegunigalsidase alfa has been observed to be stable throughout 2 weeks infusion interval

Results of the Phase I/II Clinical Trial

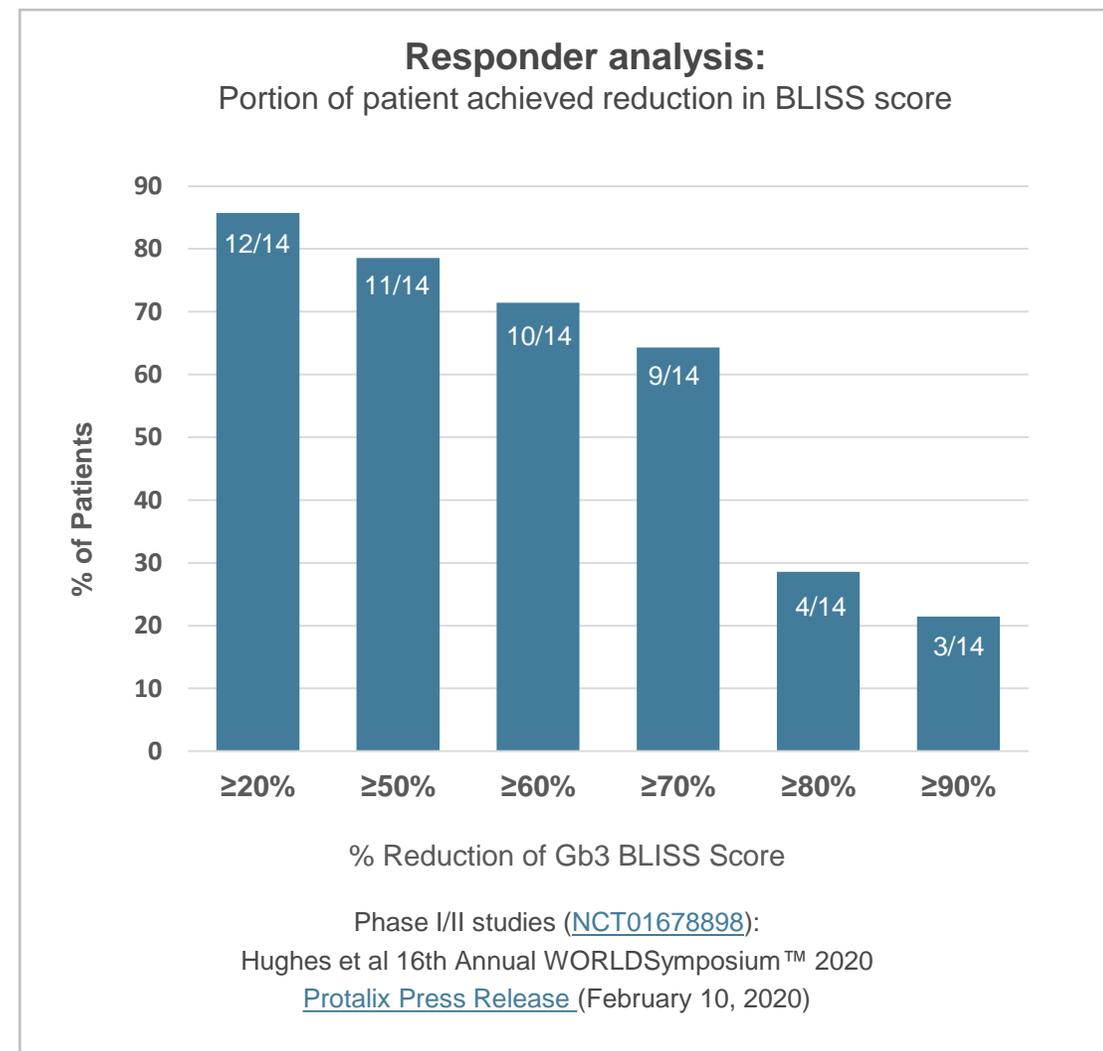
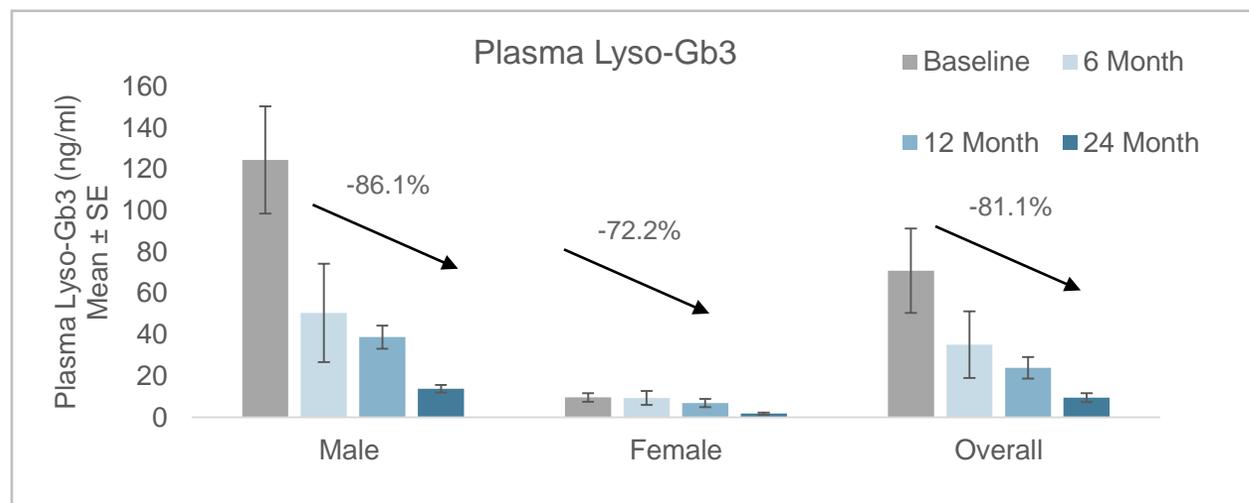
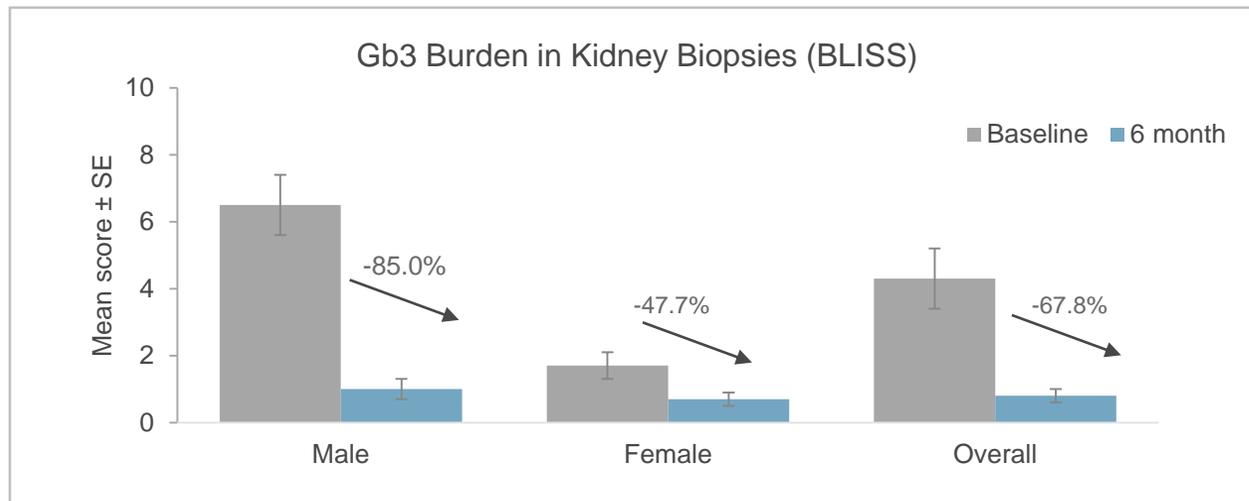


$T^{1/2}$ (hours) ~80 hours



Phase I/II Study

Substantial Reduction & High Correlation Between Two Fabry Disease Biomarkers



Pegunigalsidase alfa (PRX-102) Current Clinical Trials

| | Design | Number of Patients | Next Data Read-Out | Completed |
|---|--|-----------------------------|--|---|
|  <p>balance a Fabry clinical study</p> | <p>1mg / kg 2 weeks Randomized Double Blind Head-to-Head vs. Fabrazyme® 24 mos.</p> | <p>78 100% Enrolled</p> | <p>Interim results follow up expected H1 2021 (basis for EMA MAA Submission)</p> | |
|  <p>bridge a Fabry clinical study</p> | <p>1mg / kg 2 weeks Open Label Switch Over from Replagal® 12 mos.</p> | <p>22 100% Enrolled</p> | <p>Final results reported Q4 2020</p> |  |
|  <p>bright</p> | <p>2mg / kg 4 weeks Open Label Switch Over from Fabrazyme® and Replagal® 12 mos.</p> | <p>30 100% Enrolled</p> | <p>Top-Line results expected Q2 2021</p> |  |

Clinical Program Overview

Pegunigalsidase Alfa PRX - 102

Mechanism of Action

- Delivered to lysosome to reduce accumulated substrate
 - Increased exposure
 - Low Immunogenicity

Phase I/II

- Safety
- Reduction of Gb3 & Lyso Gb3

bridge
a Fabry clinical study

- ❖ Safety
- ❖ eGFR slope improvement

Completed Successfully



balance
a Fabry clinical study

- ❖ Safety
- ❖ Potential Superiority

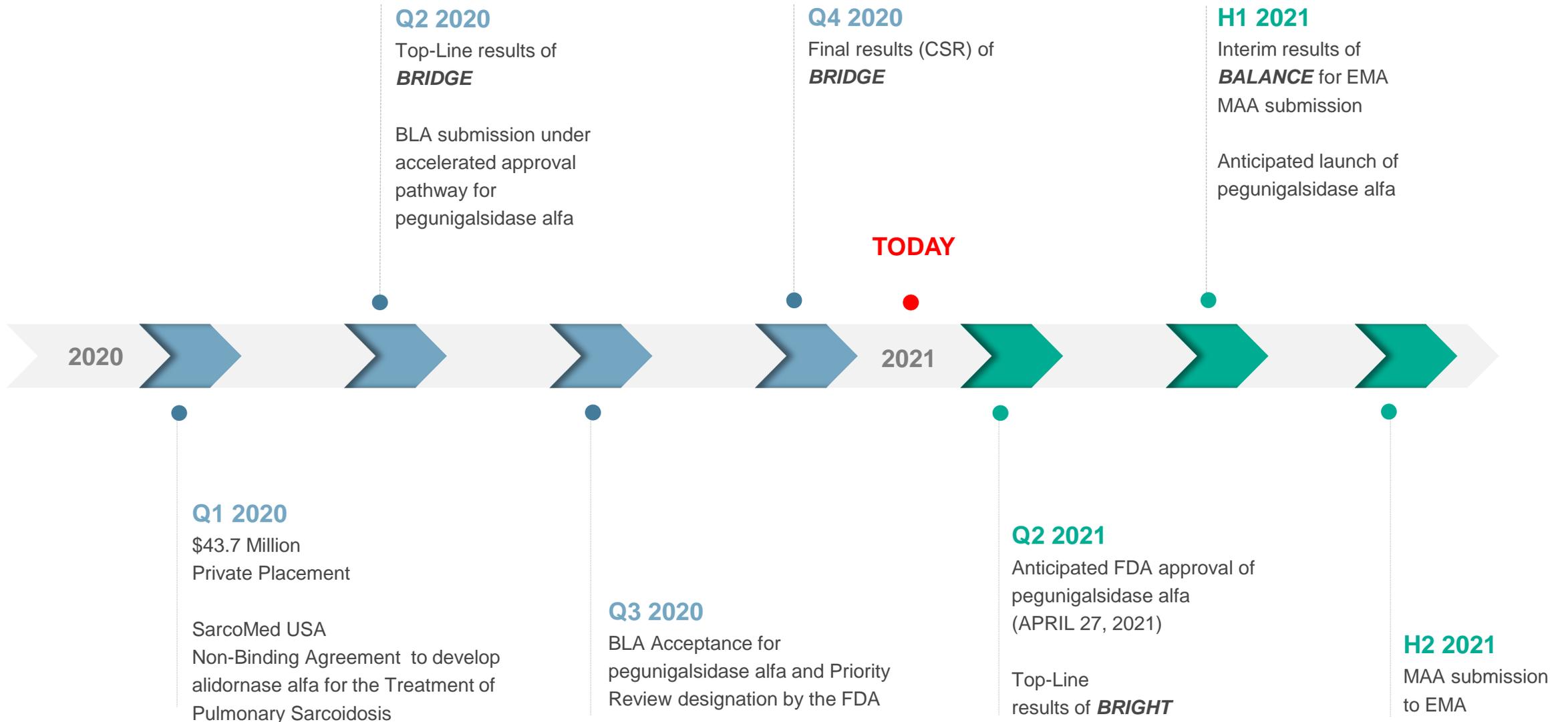
Interim Analysis
H1 2021
Final Outcomes
H1 2022

bright

- ❖ Safety
- ❖ Once every 4 weeks treatment

Topline Results
March 2022

Protalix Upcoming Expected Milestones



Committed Commercial Partner



Global Partnership with Chiesi Farmaceutici S.p.A.

Chiesi's 6,000 employees and ~\$2.5B in revenue (2019) provides Protalix with the strong Sales & Marketing partner to maximize Unigal's market potential (pending approval) as the centerpiece of their new strategic US based Orphan Drug division



Up to \$1+ billion in potential milestone payments



Tiered royalties of 15-35% (ex-US); 15-40% (US)



Committed global partner with a robust sales team and demonstrated expertise in marketing ERTs (while at Genzyme)



Tulinercept (OPRX-106) for Inflammatory Bowel Disease (IBD)

Tulinercept is a cutting-edge development candidate for IBD expressed via ProCellEx® for oral delivery of recombinant proteins whereby the plant cell wall increases resistance and delays to degradation as compared to proteins produced via mammalian cells and allows for a natural oral administration vehicle.

OVERVIEW

- IBD is a collective reference to autoimmune inflammatory diseases of the gastrointestinal system, causing inflammation and sores on the lining of the digestive tract.
 - Treatment usually begins with anti-inflammatory medications. As the severity of the disease increases, patients are generally treated with tumor necrosis factor (TNF)-alpha inhibitors (anti-TNF), which modulate the immune response.
- The current anti-TNF therapies (infused and injected) are characterized by high immunogenicity and up to a 40% loss of response, most likely due to neutralizing antibodies.
- Anti-TNF alfa biologics currently on the market have “Black Box” safety warnings for malignancies and infections. Similarly, other mechanisms for the treatment of IBD bear serious safety precautions.

OVERVIEW OF CLINICAL TRIALS TO DATE

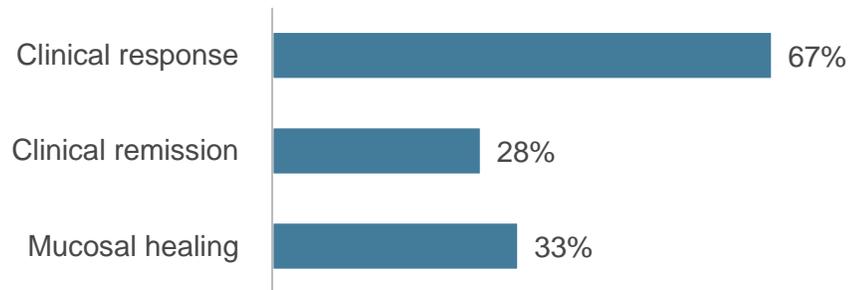
- Two clinical trials of tulinercept were completed for Ulcerative Colitis (UC):
 - **Phase I** – Healthy volunteers: safe and well tolerated.
 - **Phase IIa** – Positive results from 18 UC patients who completed the study⁽¹⁾.
- Two doses explored for induction of remission by week 8.

Sources: Colomel. *Clinical and Translational Gastroenterology*. 2016; Ben Horin. *Alimentary Pharmacology and Therapeutics*. 2011; Remicade, Humira, Simponi, Entyvio, Xeljanz prescribing information.
1. 24 patients were enrolled, 6 patients withdrew, none related to adverse events. Drop out rate consistent with other UC trials reported in similar populations.

Tulinercept (OPRX-106) for IBD

CLINICAL RESULTS FROM PHASE IIA

- 89% of patients experienced improvements in Mayo Score; 72% improved rectal bleeding; 72% improvement in fecal calprotectin and 61% improvement in Geboes score.
- Well tolerated; adverse events (AEs) were mild to moderate and transient.



- No systemic exposure of the drug was detected
- No anti-drug antibodies were detected

POSITIONING & UPSIDE

- Anti-TNF alfa mechanism – known and established first line treatment for steroid refractory and lack of response to 5-ASA.
- Low likelihood for loss of response due to lack of immunogenicity.
- Local activity in the gut and lack of systemic exposure translates to a better safety profile and removes safety concerns of infections and malignancy which appear in anti-TNF and JAK-inhibitors.
- Oral therapy – convenience in-line with newer innovative therapies.

Sources: Colombel. *Clinical and Translational Gastroenterology*. 2016; Ben Horin. *Alimentary Pharmacology and Therapeutics*. 2011; Remicade, Humira, Simponi, Entyvio, Xeljanz prescribing information. 1. 24 patients were enrolled, 6 patients withdrew, none related to adverse events. Drop out rate consistent with other UC trials reported in similar populations.

Alidornase alfa (PRX-110): Actin Inhibition Resistant DNase

OVERVIEW

- Alidornase alfa is a plant cell expressed recombinant human DNase I chemically modified to resist inhibition by actin, thus enhancing enzymatic activity.
- Recombinant human DNase I enzymatically cleaves DNA, yet its activity is inhibited by actin, which is present in the blood.
- In vitro studies have shown that alidornase alfa has a highly improved catalytic efficiency and affinity to DNA, compared to DNase I, even more so in the presence of actin.
- Alidornase alfa was previously tested in Cystic Fibrosis (CF) patients with Phase IIa trial completed in 2018. Alidornase alfa was generally well tolerated in this clinical trial with no serious adverse events reported, and all adverse events that occurred during the study were mild and transient in nature.
- In human sputa samples of CF patients, alidornase alfa exhibits greater activity compared to DNase I, without actin inhibition resistance, in breaking down extracellular DNA and lowering sputum viscosity.

POSITIONING

- Biologically active and safe in humans, alidornase alfa is being developed for other indications where it might have a potential benefit.
- Long acting DNase I is being developed for undisclosed indications.



Uricase (PRX-115) for Refractory Gout

PRX-115 is a PEGylated enzyme expressed via ProCellEx[®] for intravenous (IV) administration of recombinant Uricase, designed for safe and efficacious treatment of refractory gout.

Overview

- Gout is the most common form of chronic inflammatory disability, generally caused due to underexcretion of uric acid, leading to urate crystal deposition
- Chronic refractory gout is a painful and severe form of inflammatory arthritis that can lead to bone grinding and joint deformities resulting in loss of function and disability
- The only marketed recombinant Uricase for the treatment of refractory gout (Krystexxa[®]; Pegloticase) has a “Black Box” safety warnings for anaphylaxis and infusion reactions, mediated by strong immunogenicity
- 89% of refractory gout patients treated by Krystexxa[®] develop an immunogenic response that is associated with a failure to maintain normalization of serum uric acid levels over a 6-month therapy cycle¹
- PRX-115 is a recombinant Uricase aiming to lowering uric acid levels while having low immunogenicity and increase half-life in the circulation

Proprietary PEGylated Uricase:

- Preliminary data from pre-clinical studies demonstrate:
 - Elongated half-life in the circulation following repeated injections, indicating potential to improve pharmacokinetic profile compared to current treatment
 - Substantially low anti-drug antibodies (ADA) developed following repeated injections, indicating the potential for low immunogenic risk
 - Effective in lowering uric acid (UA) levels
- PRX-115 development plan goal is to initiate toxicity studies in Q1 2022

¹ Efficacy and Tolerability of Pegloticase for the Treatment of Chronic Gout in Patients Refractory to Conventional Treatment [JAMA. 2011 Aug 17;306(7):711-20]



Long acting (LA) DNase I (PRX-119) for NETs related diseases

PRX-119 is a cutting edge PEGylated recombinant human DNase I, expressed via ProCellEx[®], designed to elongate half-life in the circulation for NETs related diseases.

Overview

- Neutrophil extracellular traps (NETs) are web-like structures, released by activated neutrophils that trap and kill a variety of microorganisms. NETs are composed of DNA, histones, antimicrobial and pro-inflammatory proteins
- Excessive formation or ineffective clearance of NETs can cause different pathological effects. NETs formation was observed in various autoimmune, inflammatory and fibrotic conditions, diverse forms of thrombosis, cancer and metastasis¹
- Animal studies demonstrated that DNase treatment reduce NETs toxicity¹
- The FDA approved DNase I; Dornase alfa (Pulmozyme[®], Roche), for the treatment of cystic fibrosis (CF) patients via inhalation. However, this enzyme has demonstrated relatively short half-life in the circulation² and may not be effective in treating NETs related diseases
- Protalix has developed a long-acting DNase I, with an elongated half-life in the circulation for the treatment of NETs related diseases

Proprietary PEGylated DNase:

- Protalix is developing LA-DNase I, potentially to customize the treatment for various medical conditions in which NETs are involved
- Protalix's proprietary modified DNase I design for long and customized systemically circulating in the bloodstream, may potentially enable effective treatment of acute and chronic conditions
- Different animal models involving NETs pathologies, including sepsis and cancer metastasis, will be tested in collaboration with leading academics institutions

¹ Neutrophil extracellular traps in immunity and disease. Papayannopoulos V. Nat Rev Immunol. 2018 Feb;18(2):134-147

² Pharmacotoxicological expert report Pulmozyme rhDNase Genentech, Inc. Green J D. Hum Exp Toxicol. 1994 May;13 Suppl 1:S1-42



Well Capitalized with Strong Institutional & Banking Support

CASH

\$41M (3Q'20)

FINANCING

Recent ~\$44M financing (1Q'20)

CASH RUNWAY

Cash Runway to 2Q'22

EQUITY OPPORTUNITIES

Executed \$30M At-the-Market Equity Facility w/ BofA (3Q'20)

REVENUE

\$12M in revenue (3Q'20)

NET BURN RATE

\$6M/Q, declining as patients shift over to extension study

DEBT

\$58M in debt (Convertible Notes) due Nov. 2021

SHAREHOLDERS BASE

Strong Institutional shareholder base



Experienced Leadership Team



DROR BASHAN
President & CEO



Mr. Bashan has served as our President and Chief Executive Officer since June 2019. He has over 20 years of experience in the pharmaceutical industry with roles ranging from business development, marketing, sales and finance, providing him with both cross regional and cross discipline experience and a deep knowledge of the global pharmaceutical and health industries



EINAT BRILL ALMON, PH.D.
SVP, Chief Development Officer



Dr. Almon joined Protalix in Dec 2004 as a Senior Director and became our Senior Vice President, Product Development. She has many years of experience in the management of life science companies and projects including biotechnology and agrobiotech, with direct experience in clinical, regulatory, device and scientific software development, as well as a strong background and work experience in intellectual property.



EYAL RUBIN, CPA
SVP & CFO



Mr. Rubin has served as our SVP and Chief Financial Officer since September 2019. He brings to Protalix over 20 years of finance and capital markets experience, an extensive background in financial planning and operations, management and strategy and a deep knowledge of the biotechnology and pharmaceutical industries. Prior to Protalix, he served as EVP and CFO of BrainStorm Cell Therapeutics Inc., where he was responsible for corporate finance, accounting and investor relations activities.



YARON NAOS
SVP of Operations



Mr. Naos joined Protalix Ltd. in 2004 as a Senior Director for Operations and became our SVP, Operations. He has a wealth of hands-on experience and knowledge in the field of pharmaceutical development. Prior to Protalix, he served for a decade as R&D Product Manager at Dexxon Pharmaceutical Co., one of Israel's largest pharmaceutical companies, where he was responsible for technology transfer from R&D to production, and R&D activities that led to the commercialization of products.



YAEL HAYON, PH.D.
VP of R&D



Dr. Hayon, brings to Protalix over a decade of experience in pharmaceutical research in development, both in the scientific operations and the administrative functions. She most recently served as VP of Clinical Affairs of Syqe Medical Ltd. Prior to her role at Syqe Medical, Dr. Hayon held positions at LogicBio Therapeutics, Inc. and Stem Cell Medicine Ltd., Dr. Hayon holds a Ph.D. in Neurobiology & Hematology, and an MS.c. in Neurobiology, Hebrew University Faculty of Medicine, Israel.

Pegunigalsidase alfa (PRX-102) for Fabry Disease



Pegunigalsidase alfa (PRX-102)

Chemically modified PEGylated plant recombinant alpha-Galactosidase-A designed to provide active and stable enzyme throughout infusion interval for a potentially improved clinical benefit.



Current data have shown

Demonstrated substantial improvement in renal function following switchover from Replagal with a 5-point improvement in eGFR slope (Bridge Study). Circulatory half-life of ~80 hours and active enzyme throughout the infusion interval vs. 2 hours for other ERTs. Targeted enzyme activity in organs affected by Fabry disease. Well-tolerated to-date; favorable immunogenicity. Reduction of globotriaosylceramide (Gb₃) burden in kidney biopsies.



Current trials ongoing in two dosing and regimens

Goal of meeting two important unmet needs:

- Demonstrating potential greater clinical benefit in renal function and other clinical and QOL measures.
- Lowering the treatment burden of bi-weekly infusions – Improving patient's QOL.