Forward-Looking Statements

This presentation includes statements that are, or may be deemed, “forward-looking statements.” In some cases, these forward-looking statements can be identified by the use of forward-looking terminology, including the terms “believes,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “could,” “might,” “will,” “should,” “approximately,” “potential” or, in each case, their negative or other variations thereon or comparable terminology, although not all forward-looking statements contain these words. They appear in a number of places throughout this presentation and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, the genetic orphan disease drug market size and its growth potential, our position and potential in the genetic orphan disease drug market, our product pipeline, the timing and cost of trials for our products or whether such trials will be conducted at all, completion and receiving favorable results of trials for our products, timing of read out of clinical trials results, regulatory action with respect to our products, our projections for funds required for the development and commercialization of our products, development of product candidates either internally or through partnership, market adoption of our products by physicians and patients, the timing, cost or other aspects of the commercialization and marketing of our products, and future sales of our products or product candidates.

By their nature, forward-looking statements and their implications, involve risks and uncertainties because they relate to events, competitive dynamics, and healthcare, regulatory and scientific developments and depend on the economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. In addition, historic results of scientific research and clinical and preclinical trials, including interim trial data, do not guarantee that the conclusions of such or future research or trials would not suggest different conclusions or that historic results referred to in this presentation would not be interpreted differently in light of additional research and clinical and preclinical trials results. Also, while we have received Fast Track and Orphan Drug Designation for certain of our product candidates, we cannot guarantee that we will be able to maintain such designations due to reasons within or outside of our control. Although we believe that we have a reasonable basis for each forward-looking statement contained in this presentation, we caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this presentation as a result of, among other factors, the factors referenced in the “Risk Factors” section of our Annual Report on Form 20-F filed with the Securities and Exchange Commission on March 29, 2016 and in any subsequent filings with the SEC. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this presentation, they may not be predictive of results or developments in future periods. Any forward-looking statements that we make in this presentation speaks only as of the date of such statement, and we undertake no obligation to update such statements to reflect events or circumstances after the date of this presentation.
1. Orphan Disease Focused Company (Neuromuscular Diseases)

2. New USA-based Global Leadership Team

3. Lead Clinical Asset in Phase 2 with Human Proof of Concept & Significant Commercial Potential in Multiple Indications*

4. Robust Pipeline for Internal Development & BD

* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.
Clinical stage biopharmaceutical company developing novel, first-in-class disease modifying therapies to treat the underlying causes of rare genetic neuromuscular diseases with high unmet medical need

Headquartered in New Haven, CT
- R&D facility in Tel Aviv, Israel

NASDAQ: ‘ORPN’

Market Cap: ~$40M (4/26/16)

Shares Outstanding: 16.3 M
- 56% owned by insiders:
  - Cofounders each own ~20%
  - Pontifax owns ~15%

Cash and investments (12/31/15)
- $19.3 million. Additional $6.7 million in gross proceeds raised in March, 2016
### Priority Focus on Trehalose Clinical Programs.

Robust Pipeline of Preclinical Assets for Internal Development or BD

<table>
<thead>
<tr>
<th>Platform</th>
<th>Drug Candidate</th>
<th>Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
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* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.

**Priority for internal clinical development given recent human POC data**

**Development of mPRT & Read-through platforms contingent upon funding and/or business development**
New Global Leadership Team and a Board with Significant Orphan Drug Experience

Management

Colin Foster – President, CEO & Board Member
- Former CEO Bayer Pharmaceuticals Corp. and Region Head, North American for Bayer Pharma. Biotech company founder and entrepreneur

Warren Wasiewski, M.D. – Chief Medical Officer & VP R&D
- Board Certified Pediatric Neurologist. Former CMO & EVP R&D at Neurotrope BioScience; VP Neurology at Alexion

Terri Stevens – Chief Corporate Development Officer (CCDO)
- Former President & CCDO of Lupin; SVP & CCDO of Aptalis Pharma (now part of Allergan); VP BD&L at Novartis leading Pharma General Medicines BD&L negotiating team

Bob Cook – Chief Financial Officer
- Former CFO of MELA, Immune Pharmaceuticals, EpiCept and Pharmos. Former Managing Director at Chase Manhattan Bank

Leigh Cherry – VP Manufacturing
- Former Executive Director & Value Chain Leader at Merck with experience across small molecules, vaccines & biologics

Prof. Zohar Argov, MD – Special Medical Advisor to CEO
- Former President of the European Neurological Society

Bianca Jay – Director, Marketing
- Former Associate Director, Marketing at Shire and ViroPharma (purchased by Shire).

Board Members

Fred Price - Executive Chairman
- Former Chairman & CEO of BioMarin & Chiasma; Chairman of Omrix & Zymenex; BOD of Enobia & Pharmasset

Marlene Haffner, M.D.
- Former Director of Office of Orphan Products at FDA

Tom Dubin
- Former Alexion SVP & Chief Legal Officer

Dalia Megiddo – Co-Founder
- Managed two venture capital funds. Entrepreneur and founder of companies incl. Alcobra, Chiasma, Medingo

Udi Gilboa - Co-Founder
- Founder of Top Notch capital, a boutique investment bank in Israel. Entrepreneur and founder of companies incl. Alcobra, Insuline

Ran Nussbaum
- Managing Partner at Pontifax Venture Fund

Mike Burshtine
- Former Senior Partner at PWC; Biotech CFO

Isaac Krymolowski
- Former Chairman; Global Strategy – Booz Allen, Barclays Bank

Gili Cohen
- Board, academic, and investment officer experience
New Global Scientific Advisory Board

Scientific Advisory Board

**Stanley Prusiner MD – SAB Chair**
- Director, Institute for Neurodegenerative Diseases. Prof of Neurology & Biochemistry, UCSF, USA.
- Nobel Prize Laureate 1997.
- Expertise: Chronic neurodegenerative conditions.

**Zohar Argov MD**
- Medical Advisor to CEO, Bioblast Pharma. Past President of European Neurological Society.
- Expertise: Hereditary myopathies; adult onset neuromuscular disorders.

**Robert H. Brown Jr MD, PhD**
- Expertise: ALS (discovered SOD1 gene), Neurodegeneration.

**Mitchio Hirano MD, PhD**
- Director of Houston Merritt Center for Muscular Dystrophy, Columbia University, NY.
- Expertise: Mitochondrial disorders; therapy trials.

**Paul M. Matthews MD, PhD**
- Prof of Brain Sciences, Imperial College, London UK. Past Chair of Neurology at Oxford, Past GSK Vice President for Discovery & Development in Neurology.
- Expertise: Imaging and monitoring neurotherapy.

**Massimo Pandolfo MD**
- Prof & Chair Neurology, Erasme Hospital, Brussels, Belgium.
- Expertise: Hereditary ataxias (discovered Friedreich ataxia gene).
1. Orphan Disease Focused Company (Neuromuscular Diseases)
2. New USA-based Global Leadership Team
3. Lead Clinical Asset in Phase 2 with Human Proof of Concept & Significant Commercial Potential in Multiple Indications*
4. Robust Pipeline for Internal Development & BD

* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.
Trehalose 90 mg/mL IV solution – Lead Drug Candidate in Phase 2 Clinical Development

- Naturally occurring alpha-linked disaccharide formed by an α,α-1,1-glucoside bond between two α-glucose units. Well known MOA:
  - **Protein stabilizer**: Binds to & stabilizes partially folded proteins, inhibiting formation of pathological protein aggregations.
  - **Autophagy enhancer**: Unlike the proteosomal pathway, autophagy is a cellular mechanism in which part of the lipid bilayer, likely contributed by the endoplasmic reticulum, engulfs intracellular cargo (incl. protein aggregates) sequestering it for degradation by lysosomes.

- Potentially suitable as a therapeutic in PolyA (alanine) and PolyQ (glutamine) diseases for which there are pathogenic intranuclear aggregations of misfolded proteins
  - Animal proofs-of-concept in several PolyA/PolyQ & protein aggregation diseases including:
    - ✔ Oculopharyngeal muscular dystrophy (OPMD)….PolyA/muscle-based disease
    - ✔ Spinocerebellar ataxia type 3 (SCA3)…PolyQ/nerve-based disease

- To achieve suitable plasma & intracellular concentrations in humans, trehalose cannot be taken orally.

- BioBlast’s trehalose 90mg/mL IV solution:
  - ODD* in OPMD & SCA in US/EU
  - Fast Track designation for OPMD in US
  - Patents issued & pending re. methods of use; rout of administration; formulation; manufacturing.

* ODD = Orphan Drug Designation
Preclinical Rat PK Studies* Show That Delivery of Trehalose by IV Result in Significant Muscle and Nerve Cell Concentrations

**Rats**

Mean Plasma Concentration (µg/mL) of Trehalose after IV or PO Administration in Rats

<table>
<thead>
<tr>
<th>Concentration (µg/mL)</th>
<th>Mean IV</th>
<th>Mean PO</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2</td>
<td></td>
<td></td>
</tr>
<tr>
<td>4</td>
<td></td>
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</tr>
<tr>
<td>6</td>
<td></td>
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<tr>
<td>8</td>
<td></td>
<td></td>
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<td>10</td>
<td></td>
<td></td>
</tr>
<tr>
<td>12</td>
<td></td>
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</tr>
</tbody>
</table>

**Humans**

Trehalose plasma concentration after IV infusion of 27g Trehalose to OPMD patients (N=14 patients)

**Rats**

Plasma and Muscle Concentrations of Trehalose after 1g/kg IV Administration

<table>
<thead>
<tr>
<th>Time (hr)</th>
<th>Plasma Concentration (µg/mL)</th>
<th>Muscle Concentration (µg/g)</th>
</tr>
</thead>
<tbody>
<tr>
<td>4.00</td>
<td>4.00</td>
<td>3.50</td>
</tr>
<tr>
<td>8.00</td>
<td>3.50</td>
<td>3.00</td>
</tr>
<tr>
<td>24.00</td>
<td>2.00</td>
<td>1.50</td>
</tr>
<tr>
<td>48.00</td>
<td>1.00</td>
<td>0.50</td>
</tr>
</tbody>
</table>

**Rats**

Plasma and Brain Concentrations of Trehalose after 1g/kg IV Administration

<table>
<thead>
<tr>
<th>Time (Hr)</th>
<th>Plasma Concentration (ug/mL)</th>
<th>Brain, ug/g</th>
</tr>
</thead>
<tbody>
<tr>
<td>4.00</td>
<td>6.00</td>
<td>5.00</td>
</tr>
<tr>
<td>8.00</td>
<td>4.00</td>
<td>3.00</td>
</tr>
<tr>
<td>24.00</td>
<td>1.00</td>
<td>0.50</td>
</tr>
</tbody>
</table>

* Bioblast data on file
Trehalose 90mg/mL IV solution – Targeting PolyA & PolyQ Diseases with Beachhead Efforts in 2 of them (OPMD; SCA3)

- Variety of PolyA (poly-alanine) & PolyQ (poly-glutamine) diseases share a common etiology with intranuclear/intracellular disease-specific protein aggregations

<table>
<thead>
<tr>
<th>PolyA¹</th>
<th>PolyQ²</th>
</tr>
</thead>
<tbody>
<tr>
<td>✓ Oculopharyngeal muscular dystrophy (OPMD)</td>
<td>• Spinocerebellar ataxia 1</td>
</tr>
<tr>
<td>• Synpolydactyly (SPD)*</td>
<td>• Spinocerebellar ataxia 2</td>
</tr>
<tr>
<td>• Hand-Foot Genital Syndrome (HFGS)*</td>
<td>✓ • Spinocerebellar ataxia 3</td>
</tr>
<tr>
<td>• Cleidocranial Dysplasia (CCD)*</td>
<td>• Spinocerebellar ataxia 6</td>
</tr>
<tr>
<td>• Congenital Central Hypoventilation Syndrome (CCSH)</td>
<td>• Spinocerebellar ataxia 7</td>
</tr>
<tr>
<td>• Blepharophimosis/Ptosis/Epicanthus Inversus Syndrome (BPEIS)*</td>
<td>• Spinocerebellar ataxia 17</td>
</tr>
<tr>
<td>• Holoprosencephaly (HPE)*</td>
<td>• Spinobulbar Muscular Atrophy (SBMA)</td>
</tr>
<tr>
<td>• Infante Spasm Syndrome X-Linked (MR)</td>
<td>✓ • Huntington’s Disease</td>
</tr>
<tr>
<td>• X-linked Mental Retardation with Growth Hormone Deficiency (MR &amp; GH)*</td>
<td>• Dentatorubro-Pallidoluysian Atrophy (DRPLA)</td>
</tr>
</tbody>
</table>

✓ = Animal proof of concept available in literature or Bioblast data

- Bioblast focusing on two diseases, each currently in Phase 2 clinical development. Trehalose would be 1st therapeutic to treat these diseases.
  - Oculopharyngeal muscular dystrophy (OPMD) – a muscle-based disease
  - Spinocerebellar ataxia, type 3 (SCA3/Machado Joseph disease) – a nerve-based disease

* Prenatal diseases - Congenital malformations and therefore not a focus for development.
OPMD – A Rare Disease with Significant Unmet Need

PolyA disease caused by alanine trinucleotide repeats of PABPN1 protein in muscle cells leading to intranuclear PABPN1\(^2\) protein aggregations:
- Autosomal dominant disease with onset of symptoms in mid-life (i.e. ~40s-50s)

Major symptoms lead to significant morbidities in later life:
- Dysphagia (difficulty swallowing) potentially leading to aspiration pneumonia, dehydration, and severe malnutrition (cachexia)
- Tongue atrophy & speech difficulties (dysphonia)
- Upper & lower muscle weakness
- Ptosis (drooping eyelids)

No therapeutics available:
- Dysphagia & ptosis surgery
- Assistive devices

Incidence\(^1\): 1-9 per 100,000 worldwide.
- Clusters: Hispanics in South Western USA, French-Canadians, and Bukhara Jews in Israel
- Estimated 6,000 patients in USA

1. [www.orpha.net](http://www.orpha.net); [www.vencore.com/health-analytics](http://www.vencore.com/health-analytics); Youssef, S. MD, 2011 Patient Presentation; Internal data & estimates
2. PABPN1 = polyadenylate-binding nuclear protein 1
Animal Proof of Concept: Trehalose in OPMD*

Trehalose reduces the percentage of cells with inclusion bodies and with abnormal nuclei in mouse model of OPMD

Trehalose prevents deterioration in muscle strength in mouse model of OPMD

HOPEMD Phase 2 Open Label Clinical Study Design

**Locations**
- Multicenter (Israel; Canada)

**Study Design**
- Phase 2 open label clinical trial
  - First 6 months: All patients treated with 300mL of trehalose 90mg/mL IV solution weekly
  - Patients then randomized into treatment & non-treatment arms until all patient reached a minimum of 6 months treatment.

**# of Patients**
- 25 enrolled (Israel – 14; Canada –11).

**End Points**
- Safety & tolerability
- Efficacy
  - Dysphagia:
    - Penetration Aspiration Score measured by video fluoroscopy - VFS-PAS
    - Timed Cold Water Drinking test (dysphagia)
    - Swallowing Quality of Life (SWAL-QOL)
  - Muscle strength & function:
    - Range of muscle tests
HOPEMD Phase 2 Open Label Study: Dysphagia Endpoints Assessed

1. **Penetration Aspiration Score measured by Video Fluoroscopy**

<table>
<thead>
<tr>
<th>Score</th>
<th>Description of Events</th>
</tr>
</thead>
<tbody>
<tr>
<td>1.</td>
<td>Material does not enter airway</td>
</tr>
<tr>
<td>2.</td>
<td>Material enters the airway, remains above the vocal folds, and is ejected from the airway.</td>
</tr>
<tr>
<td>3.</td>
<td>Material enters the airway, remains above the vocal folds, and is not ejected from the airway.</td>
</tr>
<tr>
<td>4.</td>
<td>Material enters the airway, contacts the vocal folds, and is ejected from the airway.</td>
</tr>
<tr>
<td>5.</td>
<td>Material enters the airway, contacts the vocal folds, and is not ejected from the airway.</td>
</tr>
<tr>
<td>6.</td>
<td>Material enters the airway, passes below the vocal folds, and is ejected into the larynx or out of the airway.</td>
</tr>
<tr>
<td>7.</td>
<td>Material enters the airway, passes below the vocal folds, and is not ejected from the airway despite effort.</td>
</tr>
<tr>
<td>8.</td>
<td>Material enters the airway, passes below the vocal folds, and no effort is made to eject.</td>
</tr>
</tbody>
</table>

2. **Timed Cold Water Drinking Test**

80mL Cold Water

3. **Swallowing Quality of Life Questionnaire (SWAL-QOL)**

This questionnaire is designed to find out how your swallowing problem has been affecting your day-to-day quality of life. Please take the time to carefully read and answer each question. Some questions may look like others, but each one is different. Here’s an example of how the questions in the survey will look.

<table>
<thead>
<tr>
<th>Feel weak</th>
<th>All of the time</th>
<th>Most of the time</th>
<th>Some of the time</th>
<th>A little of the time</th>
<th>None of the time</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>1</td>
<td>2</td>
<td>3</td>
<td>4</td>
<td>5</td>
</tr>
</tbody>
</table>

Page 15
HOPEMD Phase 2 Study: Muscle Strength & Function Endpoints Assessed

**Lower Extremity Muscle Strength Tests**
1. Foot Extension
2. Knee Extension
3. Hip Flexion

**Upper Extremity Muscle Strength Tests**
4. Shoulder Abduction
5. Arm Flexion

**Muscle Function Tests**
6. Arm Lift
7. Stair Climb
8. Sit-to-Stand

**Quantitative Test**
Assessments performed using Digital hand-held dynamometer, model Hoggan MicroFET2™ manual muscle tester.
Trehalose 90mg/mL IV solution appeared safe and well tolerated.

No infusion reactions were observed.

No unexpected safety signals were reported.
  • There were no changes in laboratory parameters or physical examination, EKG, urine analysis, or insulin levels.

The most common ‘adverse event’ was glycosuria (expected).

There were 3 SAEs reported in the first 6 month protocol deemed unrelated to drug (2 aspiration pneumonia, 1 urinary tract infection, 1 sudden death).

No patient withdrew from the study.
Video Fluoroscopy Results - Per Protocol*

* Per Protocol = Canadian patients only
N=11
Cold Water Drinking Test (normal is < 8 sec)

**Intent to Treat (ITT) Population**

31.8% mean reduction in time to complete cold water drinking test vs baseline

**Minus 2 Outliers**

28.8% mean reduction in time to complete cold water drinking test vs baseline

P<0.001

* ITT = Intent To Treat and refers to all patients; N=23
Nectar and Honey Drinking Test – Different Consistencies

**Nectar Drinking Test**
- 43.8% mean reduction in time to complete nectar drinking test vs baseline

**Honey Drinking Test**
- 46.6 % mean reduction in time to complete honey drinking test vs baseline

![](chart.png)

*N=11*

**Baseline**
- Nectar: 24.2
- Honey: 29.7

**Week 4**
- Nectar: 20.0
- Honey: 26.6

**Week 8**
- Nectar: 14.9
- Honey: 17.4

**Week 12**
- Nectar: 14.7
- Honey: 14.4

**Week 16**
- Nectar: 12.7
- Honey: 13.8

**Week 20**
- Nectar: 11.0
- Honey: 11.6

**Week 24**
- Nectar: 11.7
- Honey: 12.4

*p = 0.005* for nectar, *p = 0.011* for honey
SWAL-QOL Total Symptom Scores – ITT*

12.4% mean improvement in SWAL-QOL Total Symptom Score vs Baseline

* SWAL-QOL = Swallowing Quality of Life Questionnaire
ITT = Intent To Treat and refers to all patients
N=24
Percent Change in Muscle Strength and Function Tests vs Baseline*

* N=21-22 depending on test
Trehalose 90mg/mL IV solution appeared safe and well tolerated.

No unexpected safety signals were identified. All SAEs were deemed unrelated to study drug.

Efficacy signals from 24 week analysis show the potential for improvement in dysphagia, muscle strength & muscle function.*
  - Time to consume 80mL of cold water and other liquids decreased over time.
  - VFS-PAS scores of the per protocol decreased with treatment on an individual basis.
  - Muscle power and function tests showed increase in performance.
  - SWAL-QOL scores increased.

These preliminary efficacy signals need to be confirmed in a double blind placebo controlled study to commence this year.

* Open label study was not powered for efficacy; open label studies should be cautiously interpreted.
Spinocerebellar Ataxia, Type 3 (Machado Joseph Disease) – A Rare Disease with Significant Unmet Need

**PolyQ disease caused by glutamine trinucleotide repeats in ataxin 3 protein in nerve cells leading to intranuclear ataxin 3 protein aggregations:**
- Autosomal dominant disease with onset of symptoms in early/mid-life (i.e. 30s-40s)

**Major symptoms lead to significant morbidities in later life & mortality:**
- Leads to death within ~20 years of diagnosis
- Loss of arm/leg coordinated movement; spasticity; unstable gait
- Difficulty with speech and swallowing
- Impaired eye movements
- Memory deficits

**No therapeutics available:**
- Assistive devices

**Incidence¹:**
- Estimated at 0.55/100,000 in USA/EU (diagnosed)
- More detailed analysis of prevalence currently underway

¹ [www.orpha.net](http://www.orpha.net); Internal analysis based upon published literature
Spinocerebellar Ataxia, Type 3 – Animal Proof of Concept

Trehalose significantly improves motor and coordination function in SCA3 transgenic mice

Trehalose increases cerebellum layer thickness and decreases the size of aggregates

GL, Granular layer; ML, Molecular layer; PCL, Purkinje cell layer. Scale = 50μm.
# Spinocerebellar Ataxia, Type 3 - Phase 2 Open Label Clinical Study

## Locations
- Single center: Meir Medical Center, Israel

## Status
- Phase 2 clinical trial
- Open label randomized, parallel group
- Weekly IV regimens: 15g or 30g with trehalose 90mg/mL IV solution

## # of Patients
- 14 patients enrolled.

## End Points
- Safety, tolerability; QOL
- Efficacy
  - SARA score (Scale for the Assessment And Rating of Ataxia)
  - 9 hole peg (HP); 25 foot walk (FW)
  - NESSCA score (Neurological Examination Score for Spinocerebellar Ataxia)

## Study Length
- Minimum of 6 months treatment
- Maximum of 12 months

## Current Status
- All currently treated patients anticipated to have completed first six months of therapy by early Q2, 2016.
1. Orphan Disease Focused Company (Neuromuscular Diseases)

2. New USA-based Global Leadership Team

3. Lead Clinical Asset in Phase 2 with Human Proof of Concept & Significant Commercial Potential in Multiple Indications*

4. Robust Pipeline for Internal Development & BD

* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.
Priority Focus on Trehalose Clinical Programs. Robust Pipeline of Preclinical Assets for Internal Development or BD

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<th>Drug Candidate</th>
<th>Indication</th>
<th>Preclinical</th>
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<tr>
<td>Mitochondrial Protein Replacement Therapy (mPRT)</td>
<td>BB-FA (Bioblast-Friedreich’s ataxia)</td>
<td>Friedreich’s ataxia</td>
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<td></td>
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</tr>
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Priority for internal clinical development given recent human POC data*

Development of mPRT & Read-through platforms contingent upon funding and/or business development

* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.
Mitochondrial Protein Replacement Platform (mPRT) for Mitochondrial Diseases

- A significant novel frontier in protein replacement therapy
  - ~167 mitochondrial diseases

- Proofs of concept by Bioblast/other researchers:

<table>
<thead>
<tr>
<th>Disease</th>
<th>Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Friedreich’s Ataxia</td>
<td><em>in vivo</em> &amp; cellular</td>
</tr>
<tr>
<td>Lipoamide Dehydrogenase Deficiency (LAD Deficiency)</td>
<td><em>in vivo</em> &amp; cellular</td>
</tr>
<tr>
<td>Ornithine Transcarbamylase Deficiency (OTC Deficiency)</td>
<td>cellular</td>
</tr>
<tr>
<td>Complex 1 Assembly Factor Deficiency</td>
<td>cellular</td>
</tr>
<tr>
<td>Phenylketonuria (PKU)</td>
<td>cellular</td>
</tr>
</tbody>
</table>

- Bioblast seeking to develop BB-FA for Friedreich’s ataxia & a number of other mPRT drug candidates either internally or through partnership

1. Missing protein attached to 2 delivery moieties
2. TAT penetrates membranes
3. MTS ensures arrival in mitochondria & prevents exit

1 TAT - Trans-Activator of Transcription
MTS – Mitochondrial Targeting Sequence
Friedreich’s Ataxia – A Rare Disease with Significant Unmet Need

- Autosomal recessive mitochondrial neurodegenerative disease caused by reduced function/absence of frataxin protein - a protein consequential in iron metabolism:
  - Accumulation of iron in mitochondria leads to excess production of free radicals, resulting in cell damage & death.

- Incidence\(^1\): 1 per 20-50,000 in Caucasians. Carrier prevalence of 1 per 85-110

- Major symptoms lead to significant morbidities in later life & mortality:
  - Life span is 30 to 40 years after diagnosis
  - Severe cardiomyopathy
  - Wheelchair bound

- No available therapeutics
  - Assistive devices

\(^1\) [www.orph.net](http://www.orph.net); J Md Genet 2000;37:1-8 doi:10.1136/jmg.37.1.1
## Anticipated Near-term Milestones

<table>
<thead>
<tr>
<th>Date</th>
<th>Milestone</th>
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</thead>
<tbody>
<tr>
<td>Q2, 2016</td>
<td>• SCA3 – Phase 2a open label safety study results (6 months data)</td>
</tr>
<tr>
<td>Q2, 2016</td>
<td>• Trehalose healthy volunteer dose ranging study results</td>
</tr>
<tr>
<td>mid-2016</td>
<td>• Trehalose Phase 2b double blind placebo controlled study initiation in OPMD</td>
</tr>
<tr>
<td>mid-2017</td>
<td>• Trehalose Phase 2b double blind placebo controlled study read-out in OPMD</td>
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</tbody>
</table>

Note: Bioblast, by policy, does not comment on timelines related to preclinical programs or business development initiatives
Dedicated orphan disease company in mid/late stage development with a focus on neuromuscular diseases
  • New U.S.-based global executive team & headquarters

Lead clinical drug candidate (trehalose 90mg/mL IV solution) reached Human Proof of Concept in Phase 2a open label study. Significant commercial potential in multiple indications of high unmet need*
  • First-in-class and first therapeutic to potentially treat a number of devastating PolyA/PolyQ diseases
  • Strong market exclusivity through Orphan Drug Designation & IP portfolio
  • Fast Track Designation in one program (OPMD)

Robust pipeline of drug candidates for internal development & BD
  • Animal proofs of concept established for both mPRT and read-through platforms
  • Next step development contingent upon internal funding & BD efforts


* HOPEMD Phase 2 open label study results announced in March, 2016. Open label results should be cautiously interpreted.