The Orphan Genetic Disease Company

Bioblast Pharma Ltd.
June 2016

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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.

Key Members of the Leadership Team

Fredric Price - Executive Chairman & Acting CEO

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

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

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 NPS (purchased by Shire) and Sr. Product Manager at ViroPharma (purchased by Shire)

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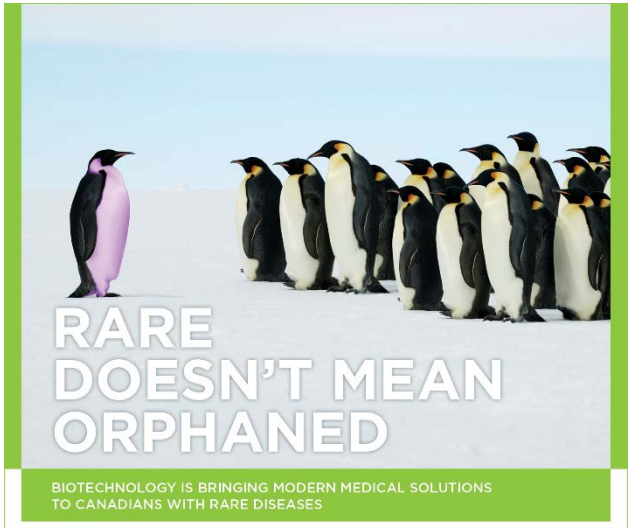
Bioblast At a Glance

- 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

- NASDAQ: 'ORPN'

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

- Cash and investments (03/31/16)
  - $21.6 million



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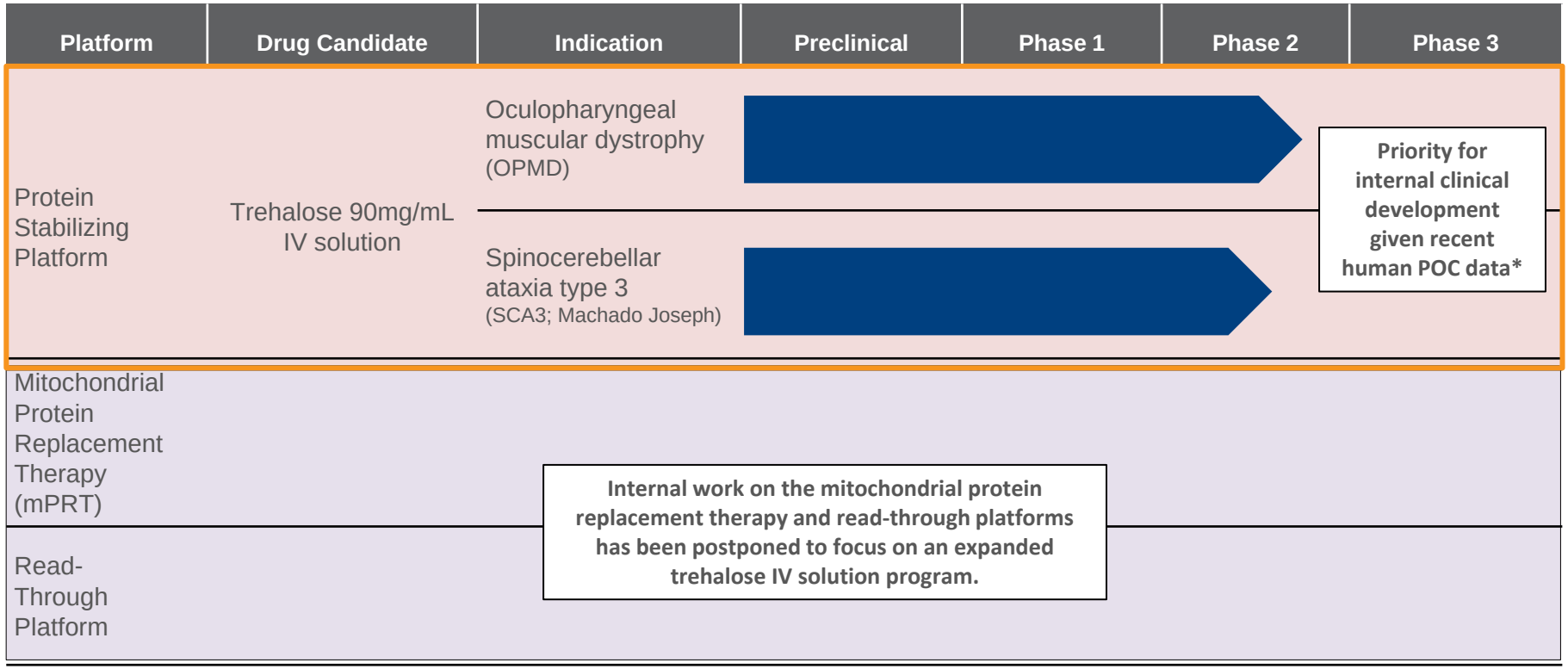
Priority Focus on Trehalose Clinical Programs



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

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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

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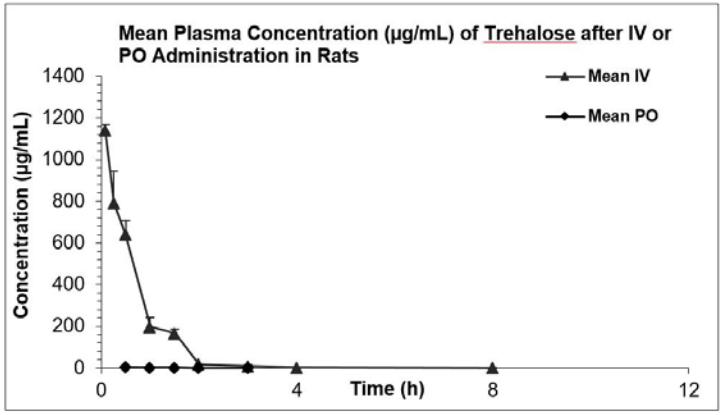
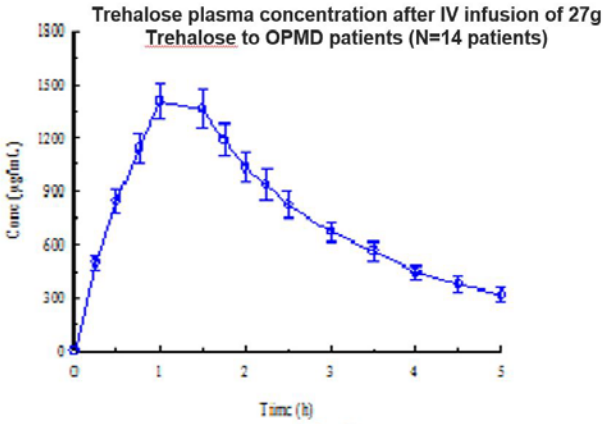
Preclinical Rat PK Studies* Show That Delivery of Trehalose by IV Result in Significant Muscle and Nerve Cell Concentrations

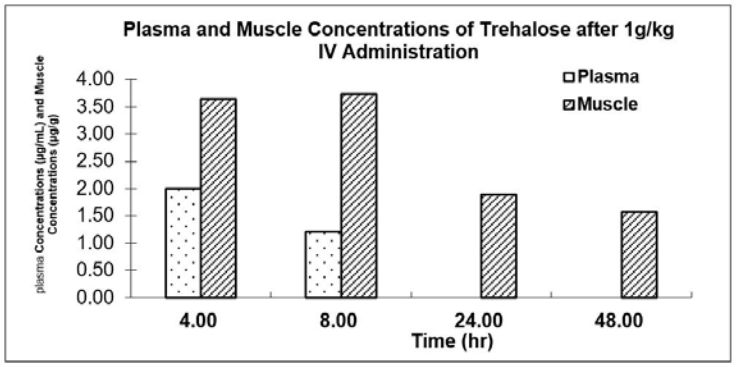
Rats



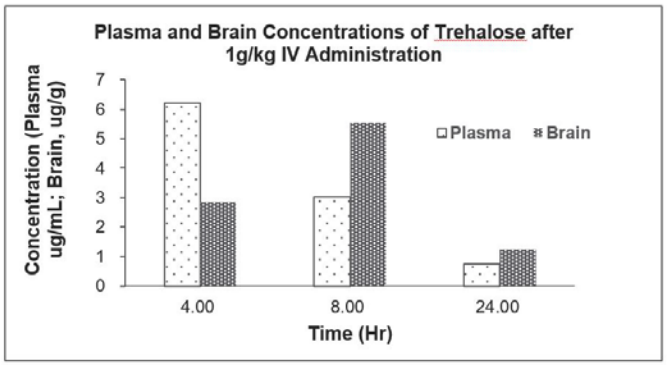
Humans



Rats



Rats



* Bioblast data on file

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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

PolyA[1]	***PolyQ[2]***
✓ • Oculopharyngeal muscular dystrophy (OPMD)	• Spinocerebellar ataxia 1
• Synpolydactyly (SPD)*	• Spinocerebellar ataxia 2
• Hand-Foot Genital Syndrome (HFGS)*	✓ • Spinocerebellar ataxia 3
• Cleidocranial Dysplasia (CCD)*	• Spinocerebellar ataxia 6
• Congenital Central Hypoventilation Syndrome (CCSH)	• Spinocerebellar ataxia 7
• Blepharophimosis/Ptossis/Epicanthus Inversus Syndrome (BPEIS)*	• Spinocerebellar ataxia 17
• Holoprosencephaly (HPE)*	• Spinobulbar Muscular Atrophy (SBMA)
• Infantile Spasm Syndrome X-Linked (MR)	✓ • Huntington's Disease
• X-linked Mental Retardation with Growth Hormone Deficiency (MR & GH)*	• Dentatorubro-Pallidoluysian Atrophy (DRPLA)

✓ = *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

[1] Brais, B. Oculopharyngeal Muscular Dystrophy: A Polyalanine Myopathy. *Current Neurology and Neuroscience Reports (2009) 9:76-82.*

[2] Zoghbi, H., Orr, H. Glutamine repeats and neurodegeneration, *Annu. Rev. Neurosci. (2000) 23:217-24.*

* Prenatal diseases - Congenital malformations and therefore not a focus for development.

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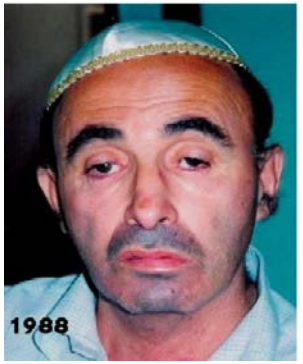
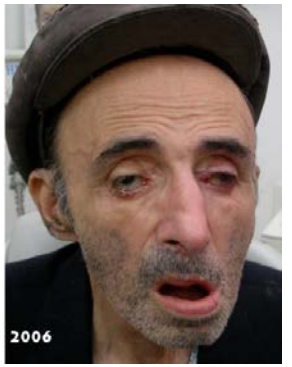
OPMD: A rare disease with a significant unmet need

OPMD Overview

- PolyA disease caused by alanine (GCN) 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; www.vencore.com/health-analytics; Youssof, S. MD, 2011 Patient Presentation; Internal data & estimates

[2] PABPN1 = polyadenylate-binding nuclear protein 1

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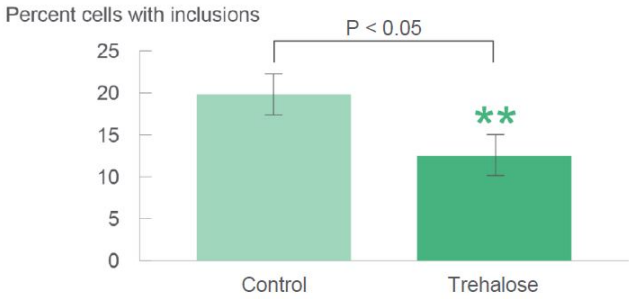
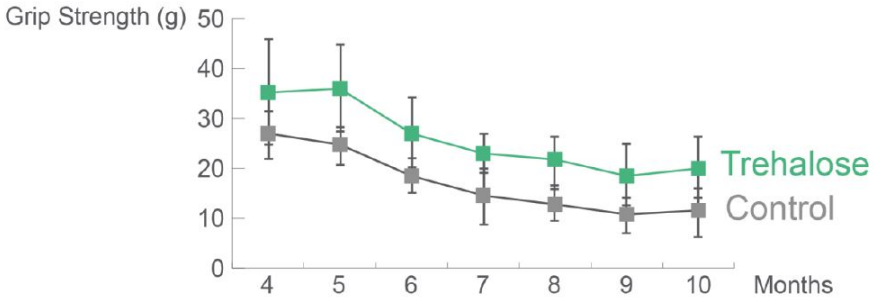
Animal Proof of Concept: Trehalose in OPMD*

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



* E. Davies, S. Sarkar, and D.C. Rubinsztein, Trehalose reduces aggregate formation and delays pathology in a transgenic mouse model of oculopharyngeal muscular dystrophy, Hum Mol Genet. 15(2006) 23-31.

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HOPEMD Phase 2 Open Label Clinical Study Design

Locations	❖ Multicenter (Israel; Canada)
Study Design	❖ Phase 2 open label clinical trial: • 6 months: All patients treated with 300mL of trehalose 90mg/mL IV solution weekly
# of Patients	❖ 25 enrolled (Israel – 14; Canada –11).
End Points	❖ Safety & tolerability ❖ Efficacy • Dysphagia: o Penetration Aspiration Score measured by video fluoroscopy - VFS-PAS o Timed Cold Water Drinking test (dysphagia) o Swallowing Quality of Life (SWAL-QOL) • Muscle strength & function: o Range of muscle tests

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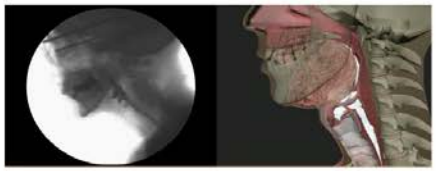
HOPEMD Phase 2 Open Label Study: Dysphagia Endpoints Assessed

1 Penetration Aspiration Score measured by Video Fluoroscopy

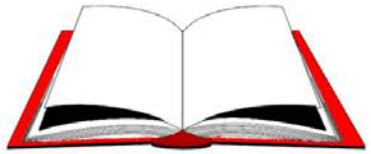


Score	Description of Events
1.	Material does not enter airway
2.	Material enters the airway, remains above the vocal folds, and is ejected from the airway.
3.	Material enters the airway, remains above the vocal folds, and is not ejected from the airway.
4	Material enters the airway, contacts the vocal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

2 Timed Cold Water Drinking Test

80mL Cold Water

3 Swallowing Quality of Life Questionnaire (SWAL-QOL)



Understanding Quality of Life in Swallowing Disorders

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.

1. In the last month how often have you experiences each of the symptoms below.

	All of the time	Most of the time	Some of the time	A little of the time	None of the time
Feel weak	1	2	(3)	4	5

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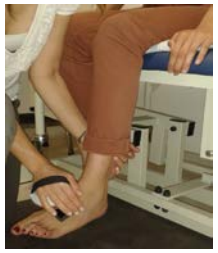
HOPEMD Phase 2 Study: Muscle Strength & Function Endpoints Assessed

Lower Extremity Muscle Strength Tests

1 **Foot Extension**



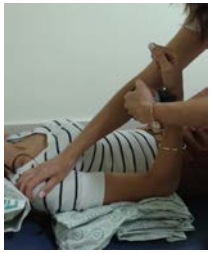
2 **Knee Extension**

3 **Hip Flexion**

Upper Extremity Muscle Strength Tests

4 **Shoulder Abduction**

5 **Arm Flexion**



Quantitative Test

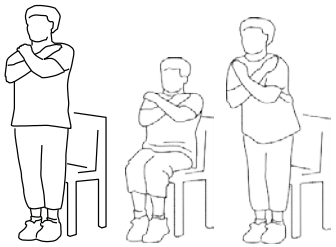
Assessments performed using Digital hand-held dynamometer, model Hoggan MicroFET2™ manual muscle tester.

Muscle Function Tests

6 **Arm Lift**

7 **Stair Climb**

8 **Sit-to-Stand**



HOPEMD Study - Safety Summary

- 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.

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HOPEMD Study - Adverse Events

Adverse events occurring in more than 10% of patients		
Adverse event	**%**	**n**
Elevated urine glucose*	52	13
Procedural pain	40	10
Back pain	28	7
Musculoskeletal pain	20	5
Headache	20	5
Site bruise	16	4
Fatigue	16	4
Nasopharyngitis	16	4
Abdominal pain	12	3
Influenza-like event	12	3
Urinary tract infection	12	3
Muscle fatigue	12	3
Myalgia	12	3
Pain in extremity	12	3
Cough	12	3

No patients withdrew due to adverse event.
Most adverse events were considered mild to moderate.
*Study-drug related

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HOPEMD Study - Serious Adverse Events

Serious adverse events		
Adverse event	**n**	**Outcome**
Urinary tract infection	1	Resolved
Aspiration pneumonia**	1	Resolved
Aspiration**	1	Death

Three adverse events occurred in 2 patients.

**Same patient

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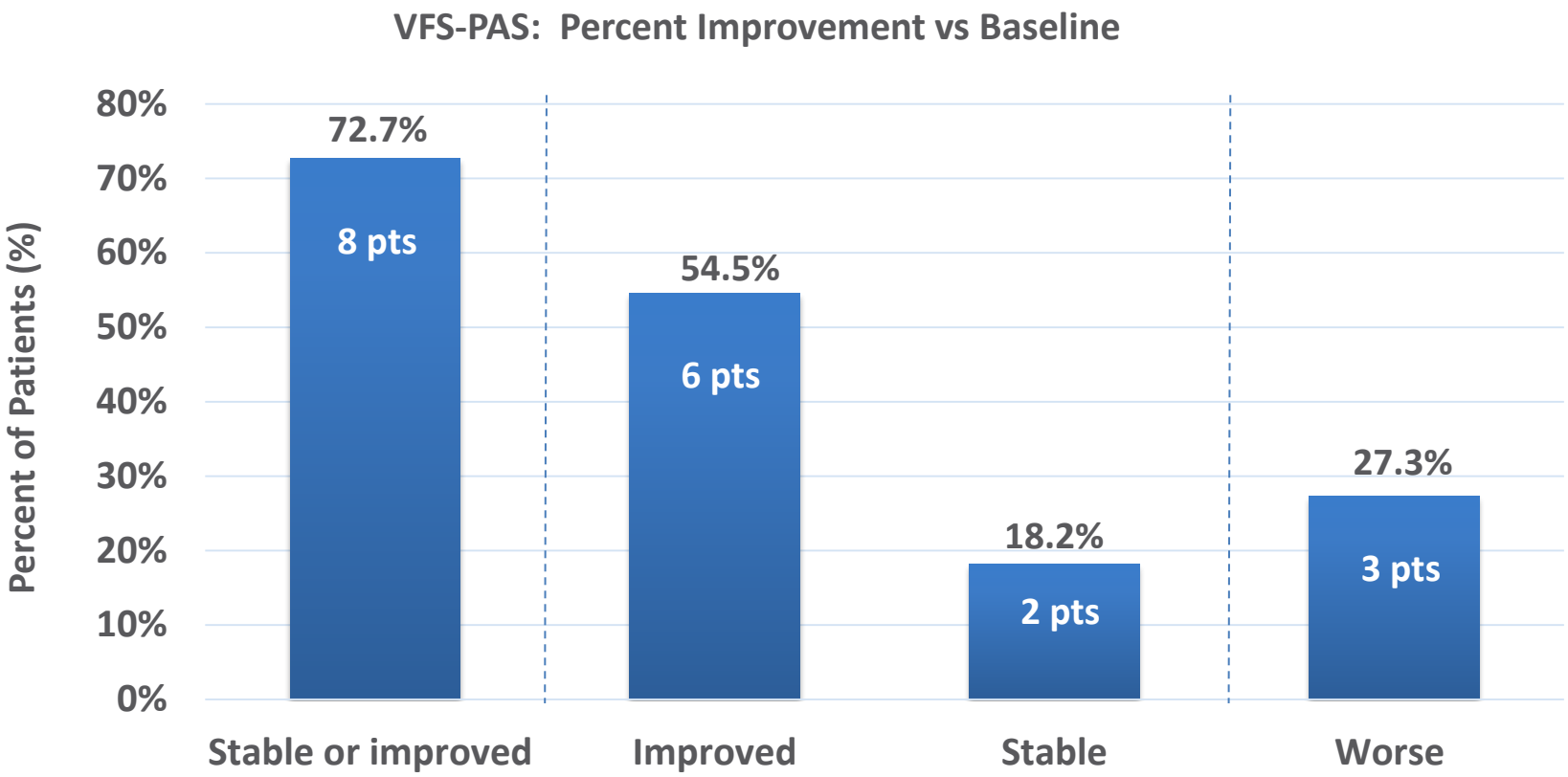
Video Fluoroscopy Results - Per Protocol*



* Per Protocol = Canadian patients only

N=11

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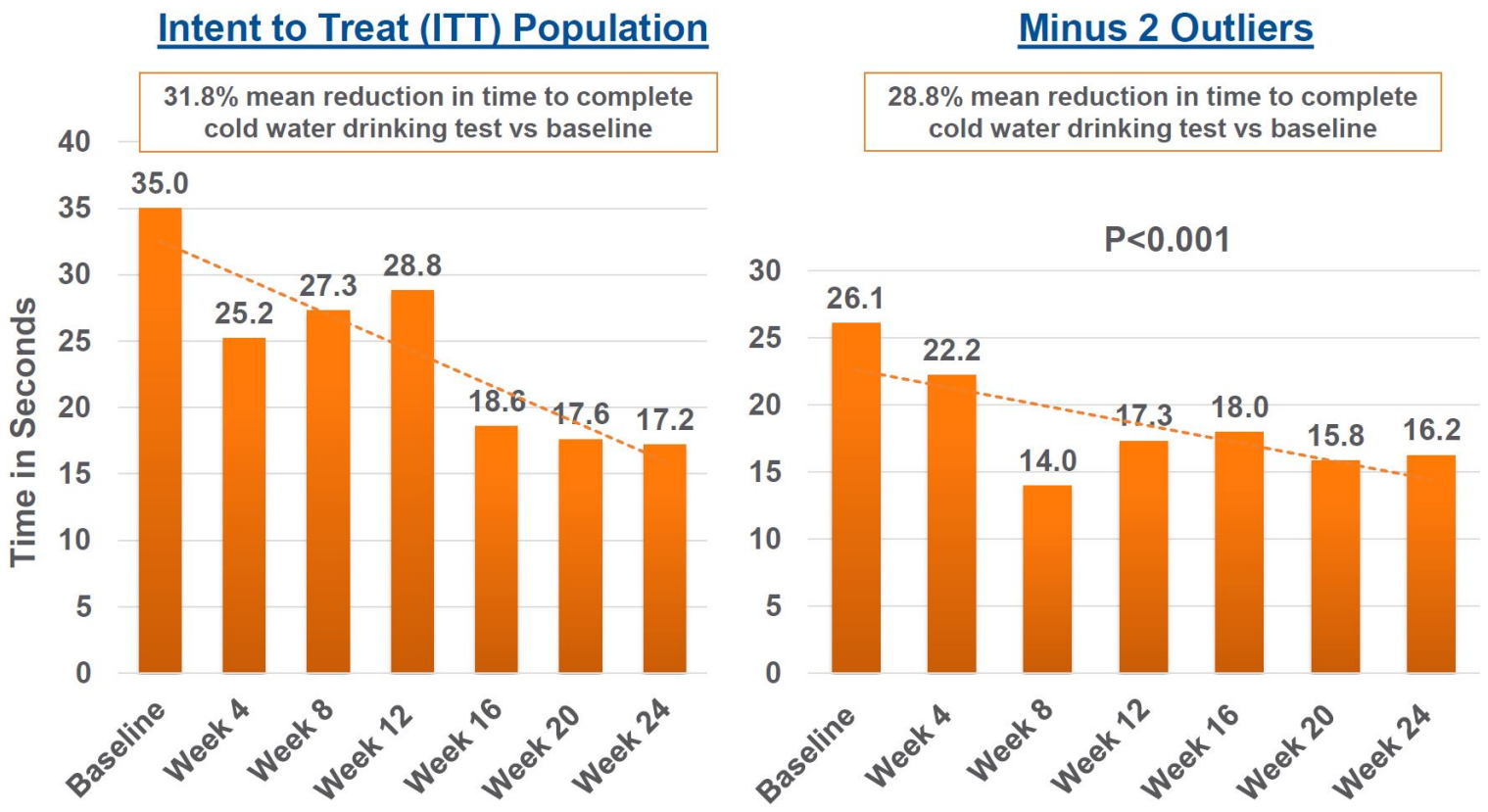
Cold Water Drinking Test (normal is < 8 sec)



* ITT = Intent To Treat and refers to all patients; N=23

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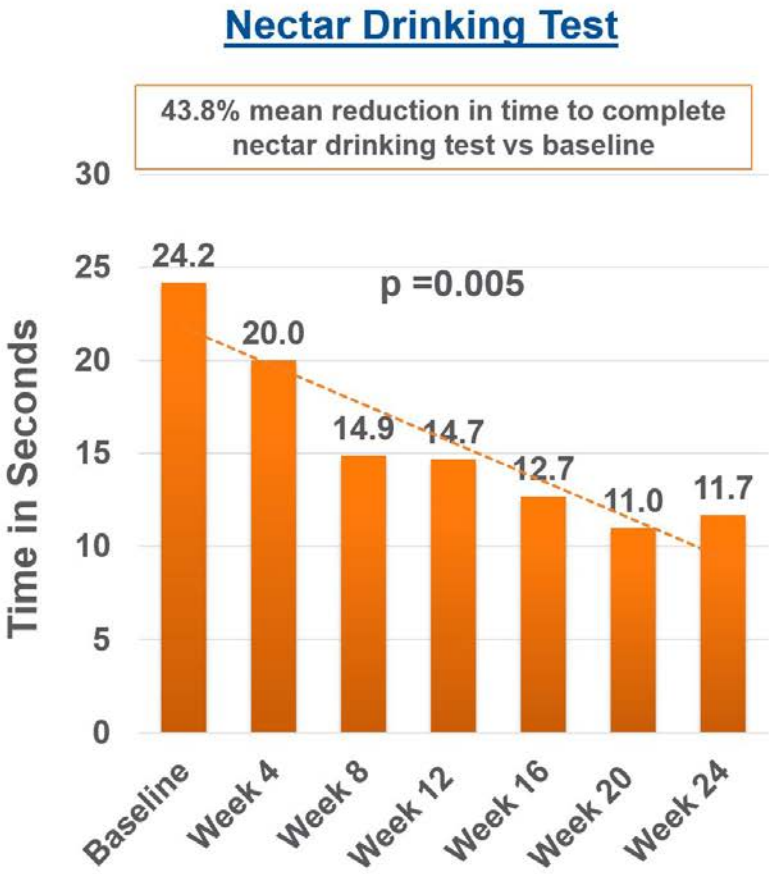
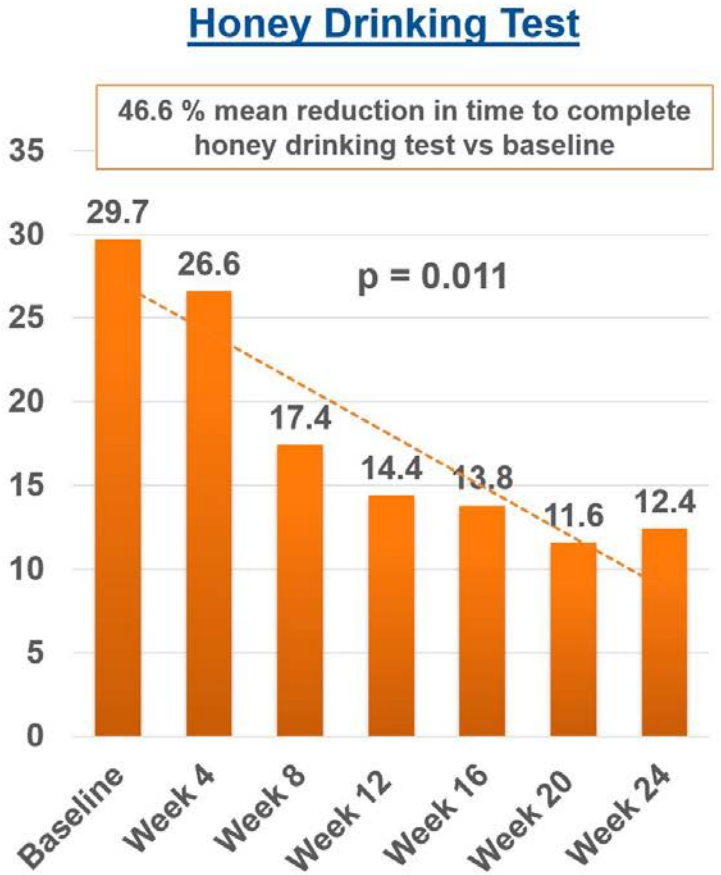
Nectar and Honey Drinking Test – Different Consistencies





N=11

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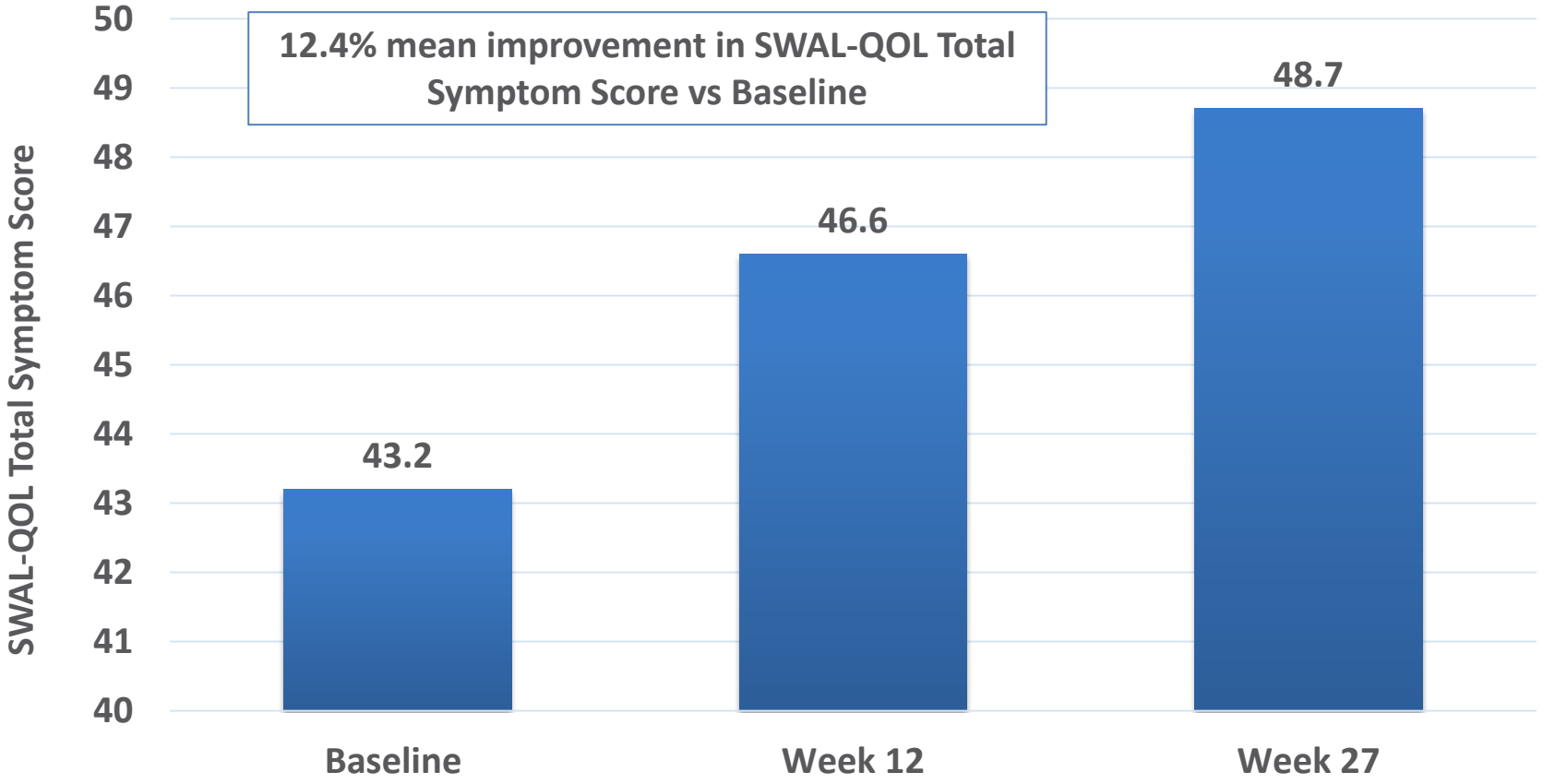
SWAL-QOL Total Symptom Scores – ITT*



* SWAL-QOL = Swallowing Quality of Life Questionnaire
ITT = Intent To Treat and refers to all patients
N=24

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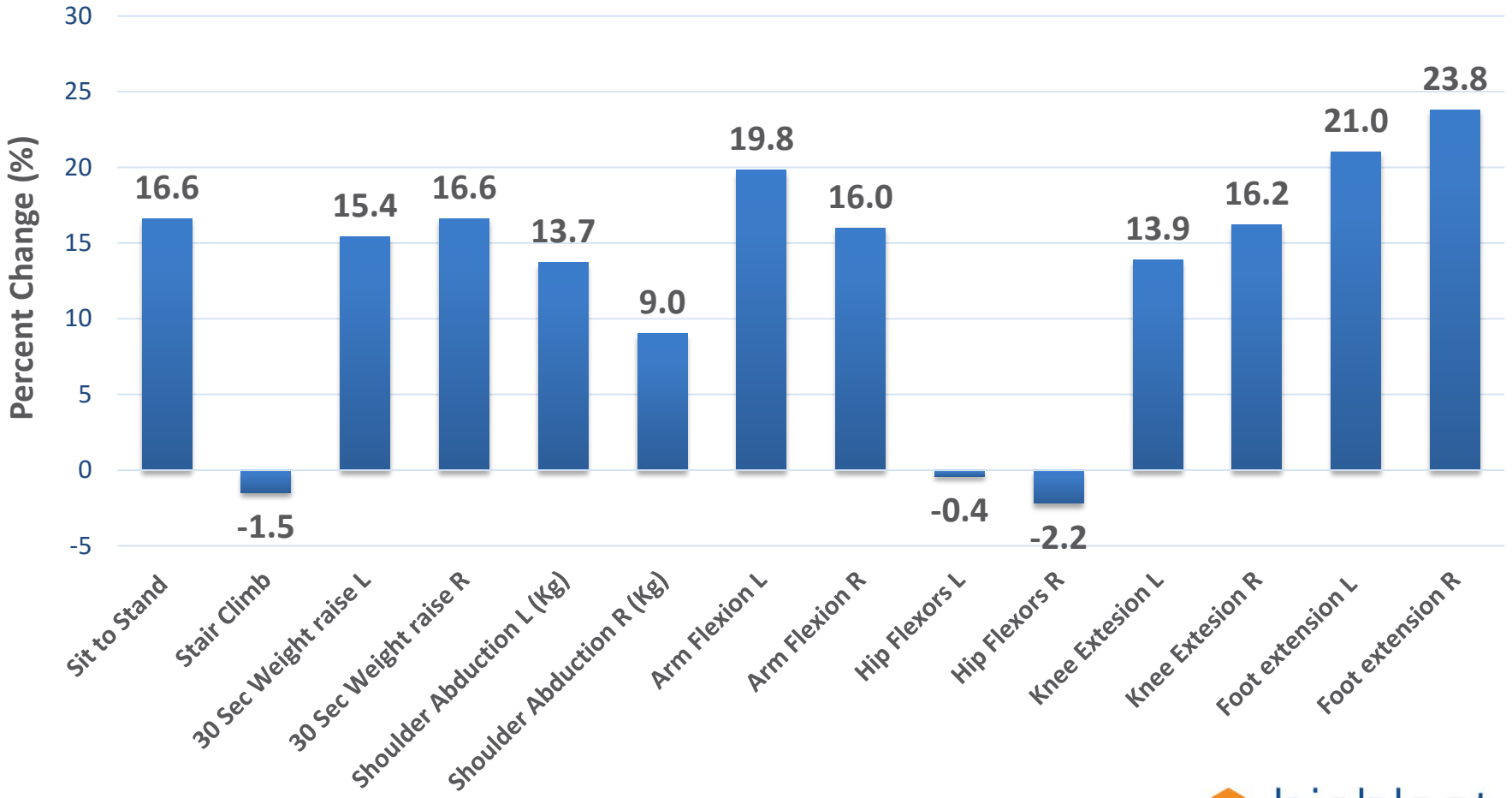
Percent Change in Muscle Strength and Function Tests vs Baseline*



*N=21-22 depending on test

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Trehalose in OPMD: Summary of 24 Week Results in Phase 2 Open Label Study*

- 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.

First pharmacological agent to show possible benefit in OPMD

* Open label study was not powered for efficacy; open label studies should be cautiously interpreted.

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Spinocerebellar Ataxia, Type 3 (Machado Joseph Disease): A Rare Disease with Significant Unmet Need

SCA3 Overview

- Poly Q 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
- Incidence[1]:
  - Estimated at 0.55/100,000 in USA/EU (diagnosed)
  - More detailed analysis of prevalence currently underway

[1] www.orpha.net; Internal analysis based upon published literature

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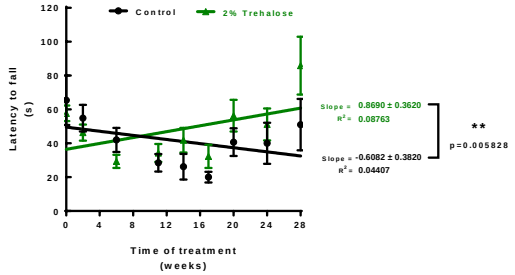
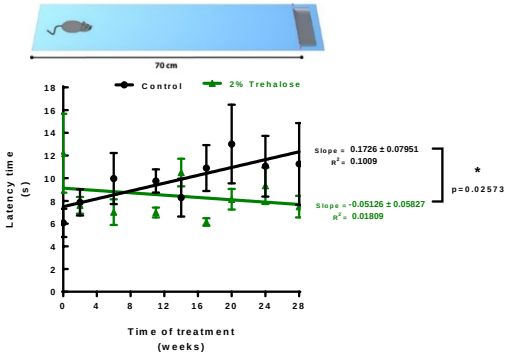
Spinocerebellar Ataxia, Type 3: Animal Proof of Concept

Trehalose significantly improves motor and coordination function in SCA3 transgenic mice

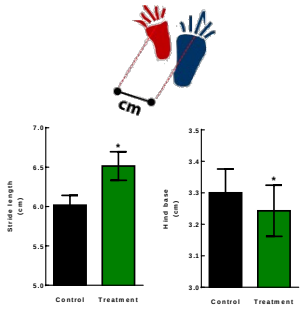
ROTAROD (STATIONARY)



SWIMMING

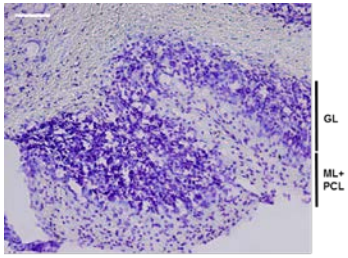


FOOTPRINT ANALYSIS

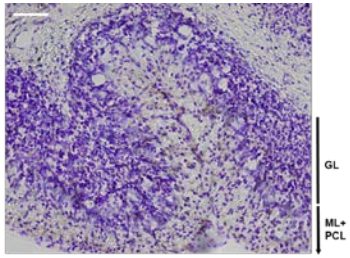


Trehalose increases cerebellum layer thickness and decreases the size of aggregates

Control

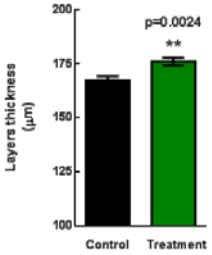


Trehalose

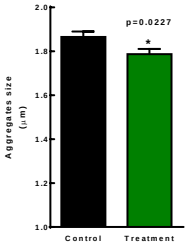


GL, Granular layer; ML, Molecular layer; PCL, Purkinje cell layer. Scale = 50µm.

LAYER THICKNESS



AGGREGATION



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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 have completed first six months of therapy.

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Investment Highlights - Summary

- **Dedicated orphan disease company in mid-stage development with a focus on neuromuscular diseases**
  - U.S.-based experienced global executive team
- **Lead clinical drug candidate (trehalose 90mg/mL IV solution) reached Human Proof of Concept in Phase 2a open label study. ***
  - First-in-class and first therapeutic to potentially treat a number of devastating PolyA/PolyQ diseases
  - Significant commercial potential in multiple indications of high unmet need
  - Strong market exclusivity through Orphan Drug Designation & IP portfolio
  - Fast Track Designation in one program (OPMD)
- **Exploring trehalose as a platform to treat multiple diseases beyond OPMD and SCA3**
  - Potential to enter into additional Phase 2 programs rapidly and with little or no incremental preclinical expenses
  - Focus on diseases in which biomarkers would help to signal early treatment effect
- **$21.6 million in cash & investments (March 31, 2016)**

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

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