
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

FORM 8-K

**CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934**

**April 24, 2017
Date of Report (Date of earliest event reported)**

ATYR PHARMA, INC.
(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-37378
(Commission
File Number)

20-3435077
(IRS Employer
Identification No.)

**3545 John Hopkins Court, Suite #250
San Diego, California 92121**

(Address of principal executive offices, including zip code)

(858) 731-8389

(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligations of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 or Rule 12b-2 of the Securities Exchange Act of 1934.

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 7.01 Regulation FD Disclosure.

On April 24, 2017, aTyr Pharma, Inc. (the “Company”) announced clinical trial data in a press release, a copy of which is furnished herewith as Exhibit 99.1.

In addition, the Company intends to use a presentation providing an update on its Resolaris program to conduct meetings at the American Academy of Neurology 69th Annual Meeting to be held April 22 – 28, 2017, and which the Company intends to place on its website. A copy of the presentation materials is furnished herewith as Exhibit 99.2. The Company does not undertake to update the presentation materials.

The information under this Item 7.01, including Exhibit 99.1 and 99.2, is being furnished and shall not be deemed “filed” for the purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, or the Exchange Act, or otherwise subject to the liabilities of that section, nor shall they be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

In connection with the announcement of clinical trial data described above, the Company announced clinical results from its Phase 1b/2 003 trial assessing the safety and potential activity of Resolaris™ in patients with early onset facioscapulohumeral muscular dystrophy (FSHD).

The Company’s exploratory trials in rare muscular dystrophies were designed to assess:

- Potential signals of clinical activity informative for developing clinical endpoints to be assessed in later-stage, placebo-controlled efficacy trials;
- Safety and tolerability of a new biologic protein, Resolaris, in patients;
- Biomarkers in these patients; and
- Data that would support further evaluation of Resolaris in rare muscular dystrophies with an immune component.

The 003 trial was an international, multi-center, open-label, intra-patient, placebo run-in, dose escalation Phase 1b/2 study designed to evaluate the safety, tolerability, immunogenicity and exploratory clinical assessments of Resolaris at weekly doses of 0.3, 1.0 and 3.0 mg/kg in patients with early onset FSHD for a total of 12 weeks. Eight patients, ages 16 to 20, participated in the study.

Potential Signals of Clinical Activity

- 63% of patients (5 of 8) had increases from baseline in their Manual Muscle Test (MMT), a validated assessment tool that measures muscle strength, with a mean change from baseline of +3.8%.
- 67% of patients measured (4 of 6) had improvement in their Individualized Neuromuscular Quality of Life (INQoL) score, a validated patient reported outcome measuring a patient’s level of disease burden. On average, patients did not have a worsening of their disease burden as measured by INQoL.

Safety and Tolerability

Resolaris was generally well-tolerated at doses up to 3.0 mg/kg once weekly in early onset FSHD. There have been no observed signs of general immunosuppression and low-level anti-drug antibody signals did not result in clinical symptoms. Adverse events were mild or moderate in intensity. There were no clinically significant changes in other safety assessments. The Company believes the observed safety results of Resolaris to date are supportive of further advancement of Resolaris.

In addition, selected slides from the corporate presentation with respect to the clinical trial data referenced above are filed herewith as Exhibit 99.3. The Company does not undertake to update the presentation materials.

Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements within the meaning of the Private Litigation Reform Act. Forward-looking statements are usually identified by the use of words such as “anticipates,” “believes,” “estimates,” “expects,” “intends,” “may,” “plans,” “projects,” “seeks,” “should,” “will,” and variations of such words or similar expressions. We intend these forward-looking statements to be covered by such safe harbor provisions for forward-looking statements and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements regarding the potential and potential therapeutic benefits of Resolaris™, the ability of the Company to successfully advance its pipeline or product candidates, undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals and the timing of such activities and development goals, the timing of initiation of additional clinical trials and of reporting results from our clinical trials, the scope and strength of our intellectual property portfolio, our ability to receive regulatory approvals for, and commercialize, our product candidates reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, risks associated with the discovery, development and regulation of our Physiocrine-based product candidates, as well as those set forth in our most recent Annual Report on Form 10-K for the year ended December 31, 2016 and in our other SEC filings. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Item 9.01 Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release of aTyr Pharma, Inc. dated April 24, 2017 (furnished herewith)
99.2	Corporate Presentation Materials of aTyr Pharma, Inc. dated April 2017, entitled “Advancing New Therapeutic Horizons for Patients with Rare Muscular Dystrophies - Resolaris Update” (furnished herewith)
99.3	Selected Slides from the Corporate Presentation Materials of aTyr Pharma, Inc. dated April 2017, entitled “Advancing New Therapeutic Horizons for Patients with Rare Muscular Dystrophies - Resolaris Update” (filed herewith)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ATYR PHARMA, INC.

By: /s/ John D. Mendlein
John D. Mendlein, Ph.D.
Chief Executive Officer

Date: April 24, 2017

INDEX TO EXHIBITS

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**IMMEDIATE RELEASE****Contact:****Mark Johnson**

Sr. Director, Investor Relations
 mjohnson@atyrpharma.com
 858-223-1163

aTyr Pharma Announces Promising Top-Line Results from Resolaris™ Phase 1b/2 Clinical Trial in Patients with Early Onset Facioscapulohumeral Muscular Dystrophy

- 63% of Patients Observed Increased Muscle Strength -

- Resolaris Demonstrated a Generally Well-Tolerated Safety Profile in Younger Patient Population -

SAN DIEGO – April 24, 2017 – aTyr Pharma, Inc. (Nasdaq: LIFE), a biotherapeutics company engaged in the discovery and development of Physiocrine-based therapeutics to address severe, rare diseases, today announced promising clinical results from its Phase 1b/2 003 trial assessing the safety and potential activity of Resolaris™ in patients with early onset facioscapulohumeral muscular dystrophy (FSHD).

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assessments. aTyr believes the observed safety results of Resolaris to date are supportive of further advancement of Resolaris.

"We would like to congratulate our patients, collaborators and team who helped us accomplish the fundamental objectives for this clinical trial," said John Mendlein, PhD, CEO of aTyr Pharma.

"We are developing Resolaris, derived from a naturally occurring protein that we believe acts on a newly discovered immunological pathway to potentially treat patients with rare muscular dystrophies characterized by immune cell imbalance," said Sanjay Shukla, MD, MS, Chief Medical Officer of aTyr Pharma. "These results are important as they reinforce previous clinical results (in adult FSHD and adult LGMD2B) with Resolaris in a younger patient population, with a potentially more aggressive progression of disease. We look forward to the advancement of Resolaris in the clinic in rare muscular dystrophies upon the identification of a pharmacodynamic assay and the successful execution of our pipeline partnering efforts."

About Resolaris™

aTyr Pharma is developing Resolaris as a potential first-in-class intravenous protein therapeutic candidate for the treatment of rare myopathies with an immune component. Resolaris is derived from a naturally occurring protein released by human skeletal muscle cells. aTyr believes Resolaris has the potential to provide therapeutic benefit to patients with rare myopathies with an immune component characterized by excessive immune cell involvement.

About Early Onset FSHD

While FSHD can manifest at any age, the onset of symptoms in many patients occurs before the age of 18. We refer to this patient population as early onset FSHD. aTyr has selected those patients with onset of symptoms before the age of ten for its current clinical trial. Within the early onset population are individuals with symptom onset at less than five years of age, with progression in disease prior to age ten. These individuals have generally the most severe muscle symptoms as well as extra-muscular manifestations including auditory deficits and retinal complications that may result in vision loss. This sub-group of early onset patients are often referred to as having "infantile onset" FSHD. Estimates of prevalence vary; however, aTyr believes the "infantile onset" FSHD population is approximately 1,000 in the U.S.

About aTyr Pharma

aTyr Pharma is engaged in the discovery and clinical development of innovative medicines for patients suffering from severe, rare diseases using its knowledge of Physiocrine biology, a newly discovered set of physiological pathways. To date, the company has generated three innovative therapeutic candidate programs based on its knowledge of Physiocrine biology in three different therapeutic areas. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 175 issued patents or allowed patent applications that are owned or exclusively licensed, including over 300 potential Physiocrine-based protein compositions. aTyr's key programs are currently focused on severe, rare diseases characterized by immune imbalance for which there are currently limited or no treatment options. For more information, please visit <http://www.atyrpharma.com>.

Forward-Looking Statements

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intend these forward-looking statements to be covered by such safe harbor provisions for forward-looking statements and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements regarding the potential and potential therapeutic benefits of Resolaris™, the ability of the Company to successfully advance its pipeline or product candidates, undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals and the timing of such activities and development goals, the timing of initiation of additional clinical trials, the scope and strength of our intellectual property portfolio, our ability to receive regulatory approvals for, and commercialize, our product candidates and of reporting results from our clinical trials reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, risks associated with the discovery, development and regulation of our Physiocrine-based product candidates, as well as those set forth in our most recent Annual Report on Form 10-K for the year ended December 31, 2016 and in our other SEC filings. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

ADVANCING NEW THERAPEUTIC HORIZONS
FOR PATIENTS WITH RARE MUSCULAR DYSTROPHIES

HARNESSING NOVEL PATHWAYS IN IMMUNOLOGY TO PROMOTE HOMEOSTASIS IN MUSCLE

ATYR PHARMA, INC.
RESOLARIS™ UPDATE
AMERICAN ACADEMY OF NEUROLOGY
APRIL 2017



Forward-Looking Statements

The following slides and any accompanying oral presentation contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” “opportunity,” or “continue,” and other similar expressions are intended to identify forward-looking statements. For example, all statements we make regarding the potential therapeutic benefits of Physiocrines and our product candidates, including Resolaris™ and Stalaris™, the ability to successfully advance our pipeline or product candidates, the timing within which we expect to initiate, receive and report data from, and complete our planned clinical trials, and our ability to receive regulatory approvals for, and commercialize, our product candidates, our ability to identify and discover additional product candidates, our projected cash expenditures, and the ability of our intellectual property portfolio to provide protection are forward-looking statements. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These risks, uncertainties and other factors are more fully described in our filings with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K and in our other filings. The forward-looking statements in this presentation speak only as of the date of this presentation and neither we nor any other person assume responsibility for the accuracy and completeness of any forward-looking statement. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

We own various U.S. federal trademark applications and unregistered trademarks, including our company name, Resolaris™ and Stalaris™. All other trademarks or trade names referred to in this presentation are the property of their respective owners. Solely for convenience, the trademarks and trade names in this presentation are referred to without the symbols ° and ™, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.



Pioneers of new therapeutic intervention points in immunology
Promoting Homeostasis



Favorable safety profile and potential clinical activity from
1st Physiocrine program, Resolaris, in 2 rare muscular dystrophies



Advancing **2nd Physiocrine** program for rare lung diseases,
Stalaris, into human trials this year



Potential **3rd Physiocrine**-based opportunity
as a 2017 IND candidate in a **3rd** therapeutic area

Pursuing partnership(s) for one or more of the above programs
to accelerate clinical and preclinical pipeline

>175 issued/allowed patents
Strong Leadership Team associated with **18** approved drugs
\$76M cash 2016 EOY

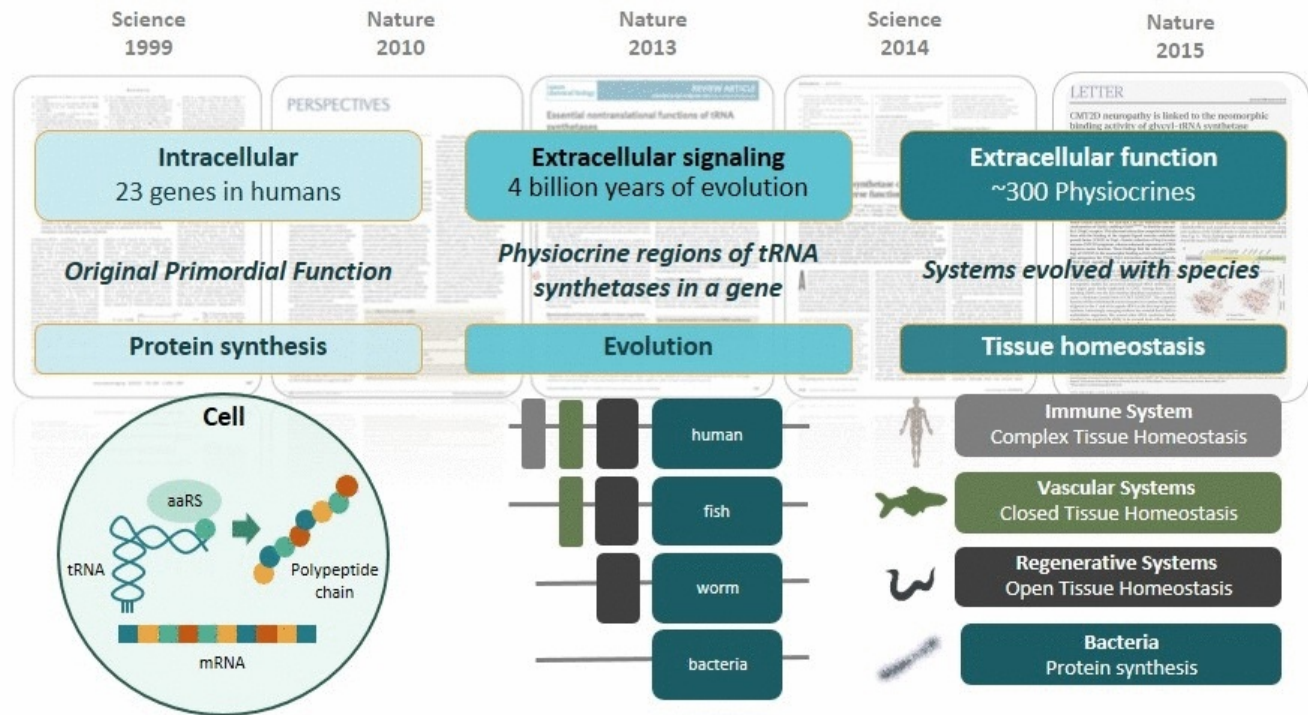
THE POWER OF PHYSIOCRINES ORCHESTRATING HOMEOSTASIS

NEW CLASS OF PROTEINS FROM ALTERNATIVE SPLICING OF ANCIENT GENES

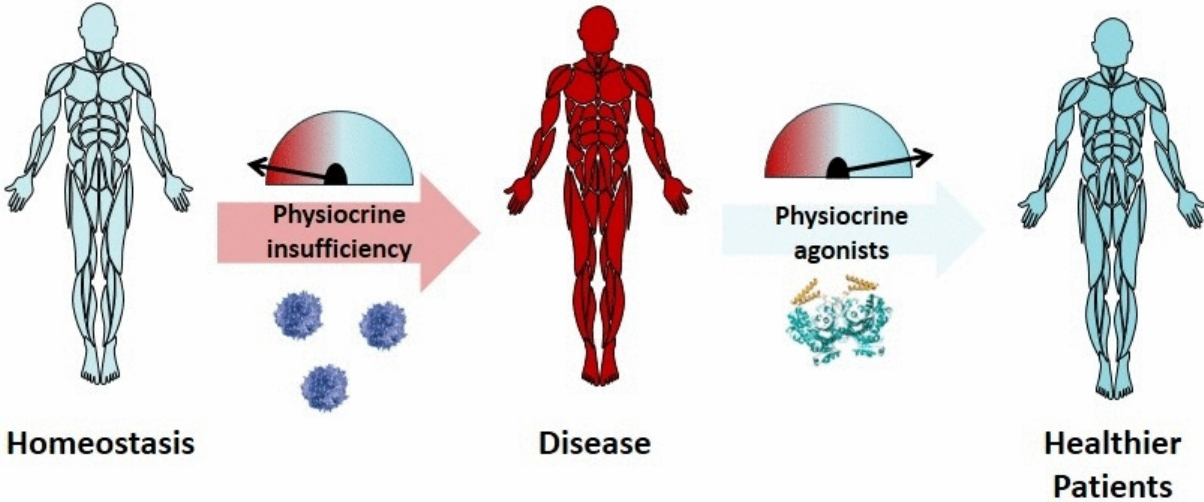


What are *Physiocrines*? Extracellular Signaling Regions

PHYSIOCRINE
BIOLOGY



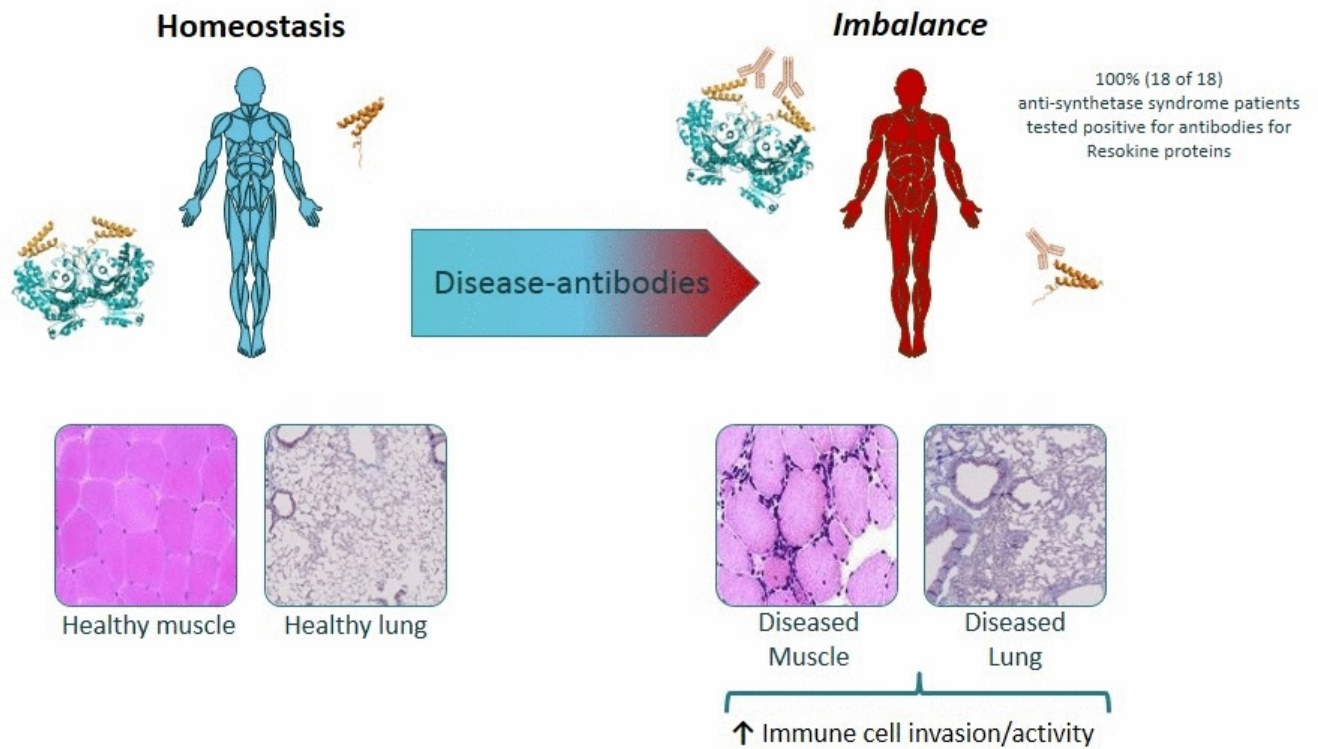
Schimmel et al. Human tRNA Synthetase Catalytic Nulls with Diverse Functions. *Science*, 2014.



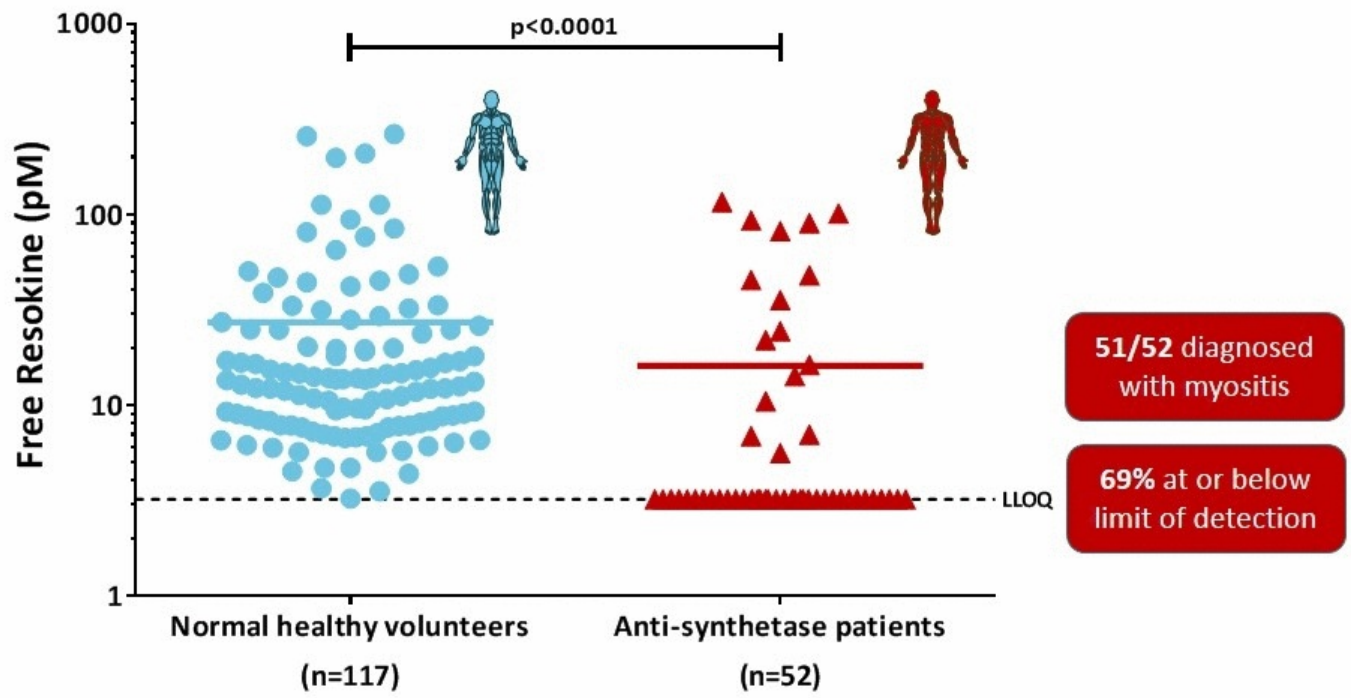
Evidence for Homeostatic Role of Resokine in Humans

Disrupting the Resokine Pathway Promotes Muscle and Lung Disease

RESOKINE
PATHWAY



Free Resokine Pathway in Anti-Synthetase Patients Diminished



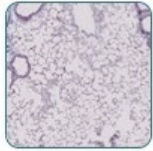
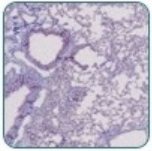


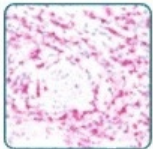
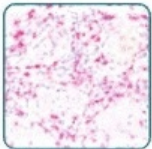


Unpublished data from aTyr and collaborator
Statistics: Mann-Whitney test

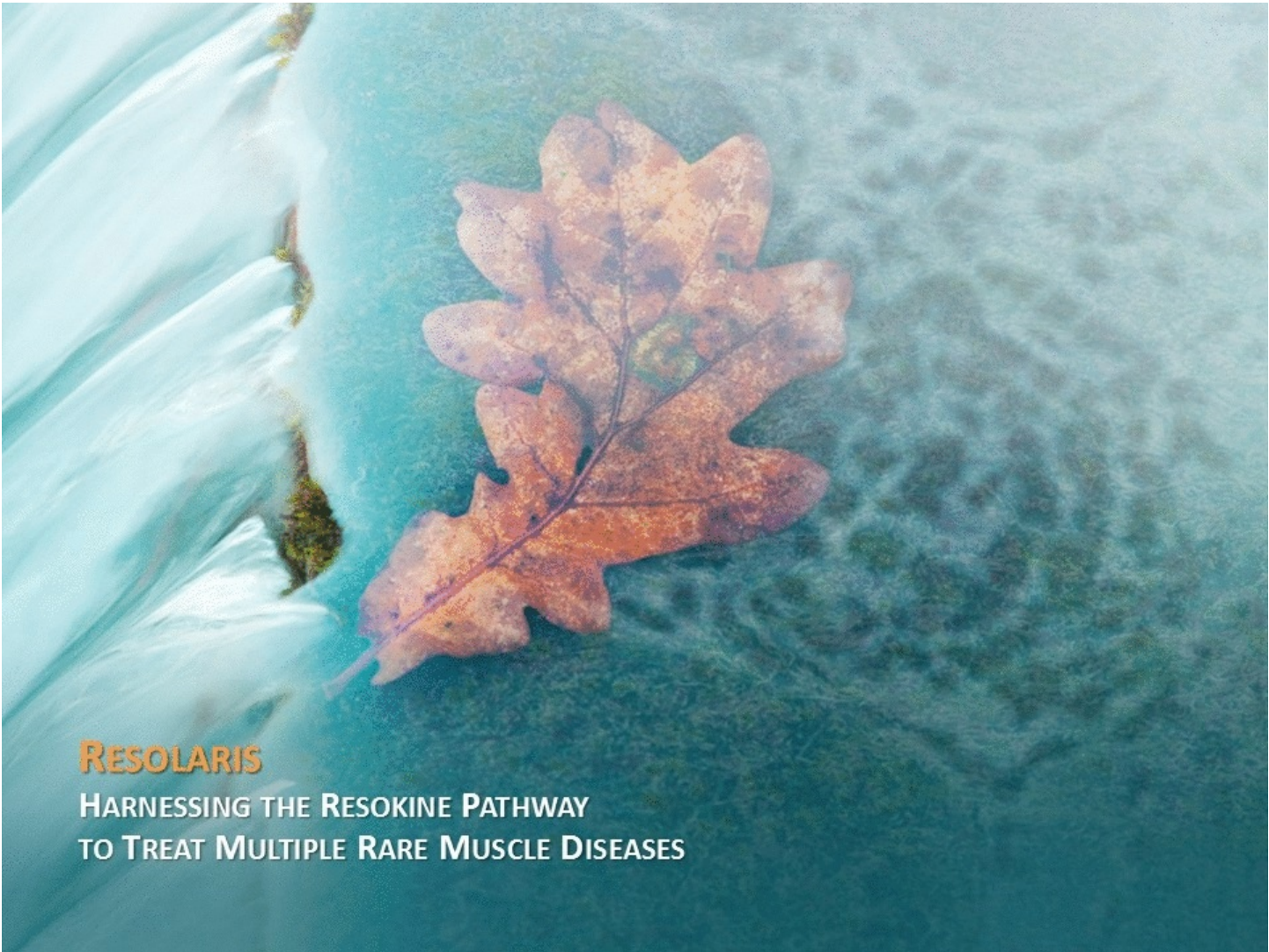
Agonists of the Resokine Pathway in Immune Driven Models

Balancing the immune response to tissue insults

RESOKINE
PATHWAY

Disease Model	Resokine Homeostatic Effect		Immune Targets	
Skeletal Muscle Statin Induced Myopathy		→		CD4/CD8 & macrophages
Lung Bleomycin Induced Lung Fibrosis		→		Th17/CD4
Colon TNBS Induced Colitis		→		Th17/CD4
Skin IL23 Induced Psoriasis		→		Th17/CD4

In vivo administration of Resokine proteins to animal models of T cell driven disease states. Cell type indicates type of cells involved but may not be limited to these cells.



RESOLARIS

HARNESSING THE RESOKINE PATHWAY
TO TREAT MULTIPLE RARE MUSCLE DISEASES



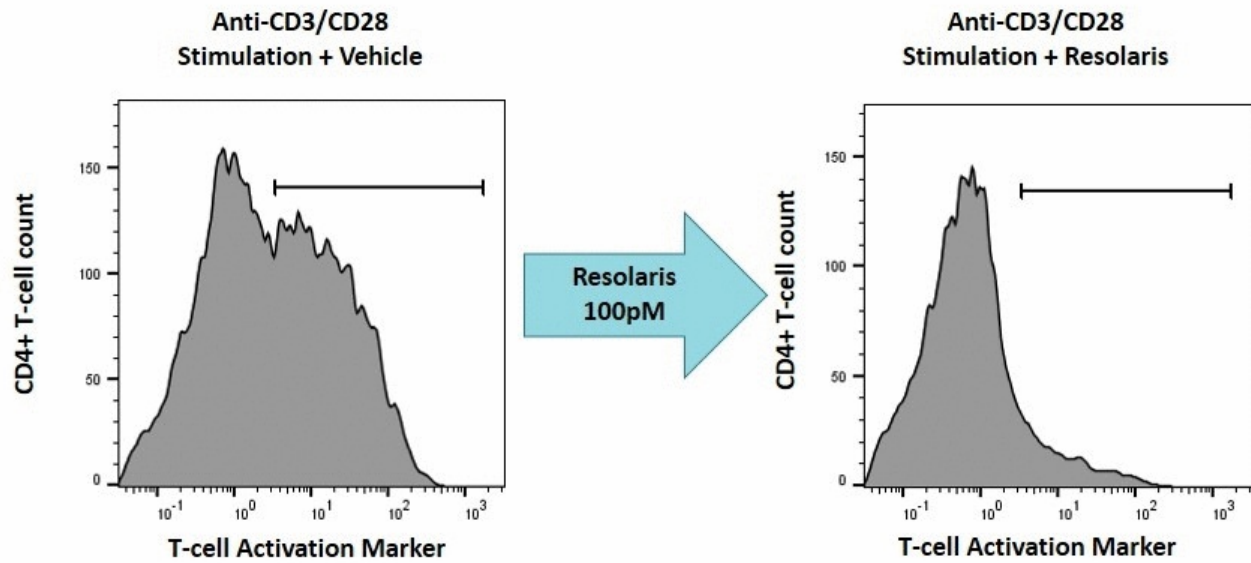
Derived from **Resokine**: a naturally occurring protein, the histidine aminoacyl tRNA synthetase (HARS)

- Skeletal muscle secretes Resokine
 - Resokine, an agonist, plays a role in homeostasis & T cell responses in muscle
 - Recombinant version of Resokine
 - Demonstrated favorable safety profile and potential clinical activity in two rare muscular dystrophy indications
 - Therapeutic potential for rare myopathies with an immune component (RMIC), **over 20** potential indications
- **Strategy:** Establish broad utility in multiple indications

Resolaris Tempers Activated T cells

Demonstrated effect as an immuno-modulator

IN VITRO
T CELL
MODULATION



Resolaris with **Activated** T-cells Promotes a More **Resting** T-cell Phenotype

On the Left: Gated on CD4⁺ T cells. Resolaris at 100 pM. 24 hours stimulation with anti-CD3/CD28 Abs.

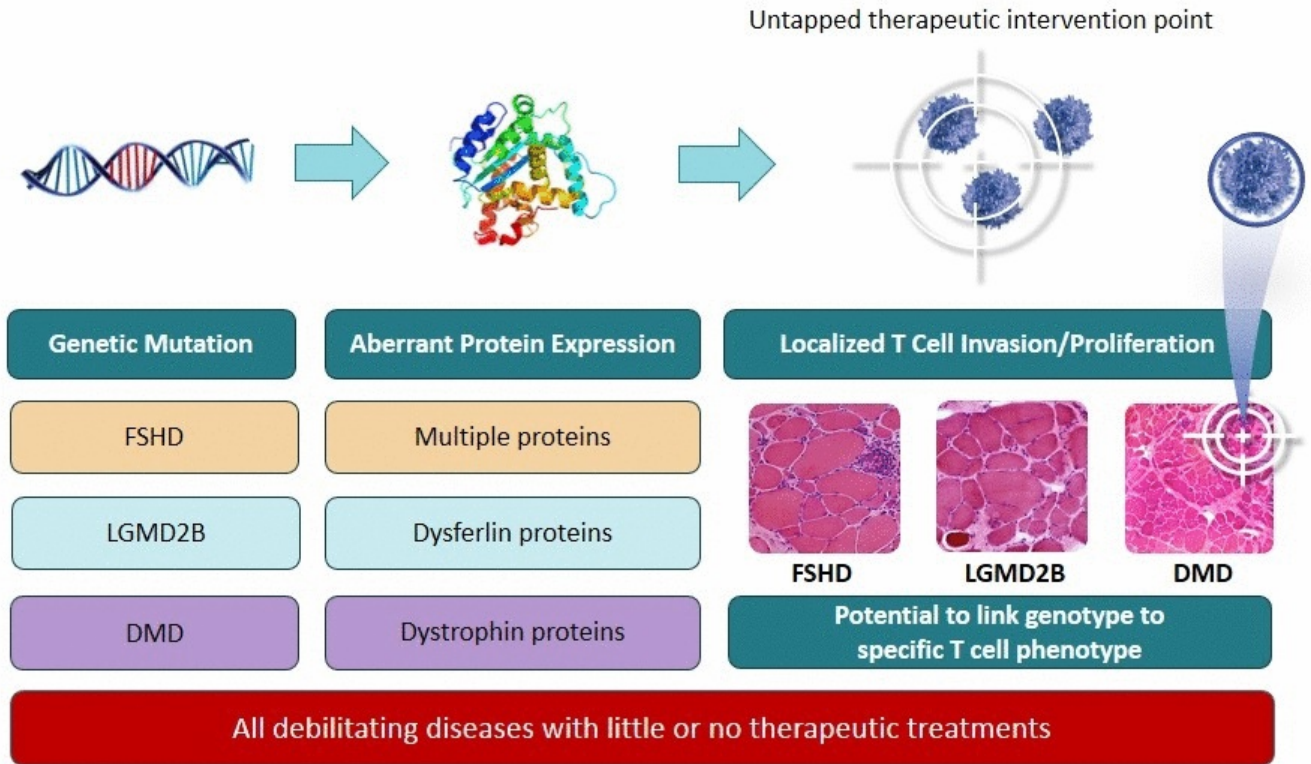
On the Right: T cells were stimulated with anti-CD3/CD28 antibodies in the presence of vehicle or Resolaris. After 24 h, supernatants were collected and analyzed by ELISA. Statistics by T test

Rare Myopathies with an Immune Component

Chronic damage, homeostasis disrupted

SHARED

PATHOPHYSIOLOGY



Frisullo et al., *J. Clin. Immunol.*, 2011. Gallardo et al. *Neurology*, 2001. Flanigan et al. *Human Gene Therapy*, 2013.

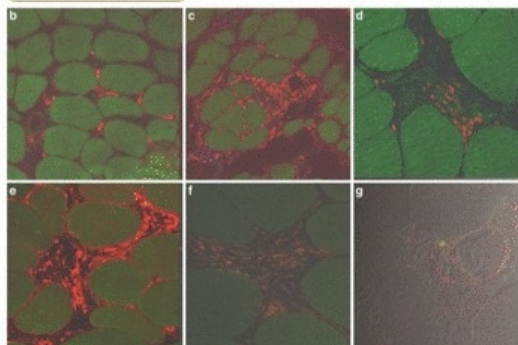
FSHD = Facioscapulohumeral Muscular Dystrophy (FSHD). LGMD2B = Limb Girdle Muscular Dystrophy 2B.

DMD = Duchenne Muscular Dystrophy.

T Cells Central to Pathophysiology of RMICs

IMMUNE
IMBALANCE

FSHD



Endomyosial infiltrates, in which CD8+ cells predominated (Fig. 2b–d), and perivascular infiltrates, mainly constituted by CD4+ cells (Fig. 2f) in all samples



LGMD2B

Cells	Polymyositis	Dysferlinopathy
CD8+	46.5 ± 10.3	11.1 ± 6.6
CD4+	27.3 ± 11.5	40.6 ± 22.8
Macrophages	27.7 ± 7.6	36.7 ± 23.7

Endomyosial mononuclear cell infiltrates in clusters (cell count per cluster)
5/12 dysferlinopathy patients originally diagnosed with inflammatory myopathy

LGMD2B & DMD

Cells	Polymyositis	Dysferlinopathy	DMD/BMD
CD8+	3.3 ± 1.8	1.3 ± 1.1	2.0 ± 1.6
CD4+	12.3 ± 6.4	5.7 ± 4.4	4.9 ± 5.7
Macrophages	10.8 ± 6.5	7.8 ± 4.3	3.7 ± 3.1

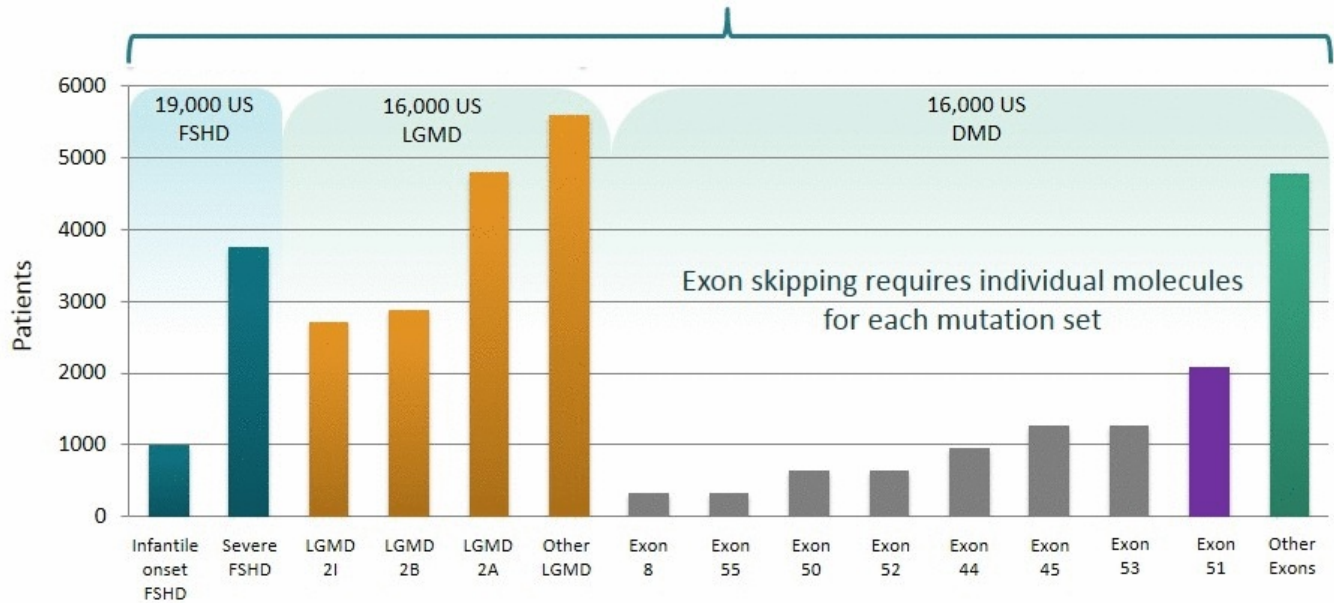
Comparison of inflammatory cells in muscle biopsy samples of dysferlinopathy, DMD/BMD and polymyositis patients (average cell count per muscle section)

Resolaris: One Product, Multiple RMICs

Promise for severely afflicted myopathy patients

MARKET
OPPORTUNITIES

Resolaris has broad potential across multiple rare myopathies



FSHD: Average prevalence rates of FSHD are approximately 1/17,000. Applying this rate to the US population based on recent census data equals approximately 19,000.
LGMD: 16,000 cases estimated in US population. 1/20,000 Wickland and Kisse, *Neural. Clin.* 20'14. Relative Prevalence of Limb Girdle Muscular Dystrophies in the United States Population. Wicklund et al., *Neurology* 2013.
DMD: Prevalence of approximately 5/100,000. Orphanet Report Series - Prevalence of rare diseases: Bibliographic data - May 2014 - Number 1

Objectives

Evaluate Safety and Tolerability

- ✓ Build safety dossier for Resolaris
- ✓ Multiple indications, different dosing regimens, longer duration

Evaluate Potential Activity Assessments*

- ✓ Functional / Strength: MMT
- ✓ Patient Reported Outcomes: INQoL
- ± MRI / Biomarkers assessments

Evaluate three potential indications: Adult LGMD2B, Adult FSHD, & Early Onset FSHD

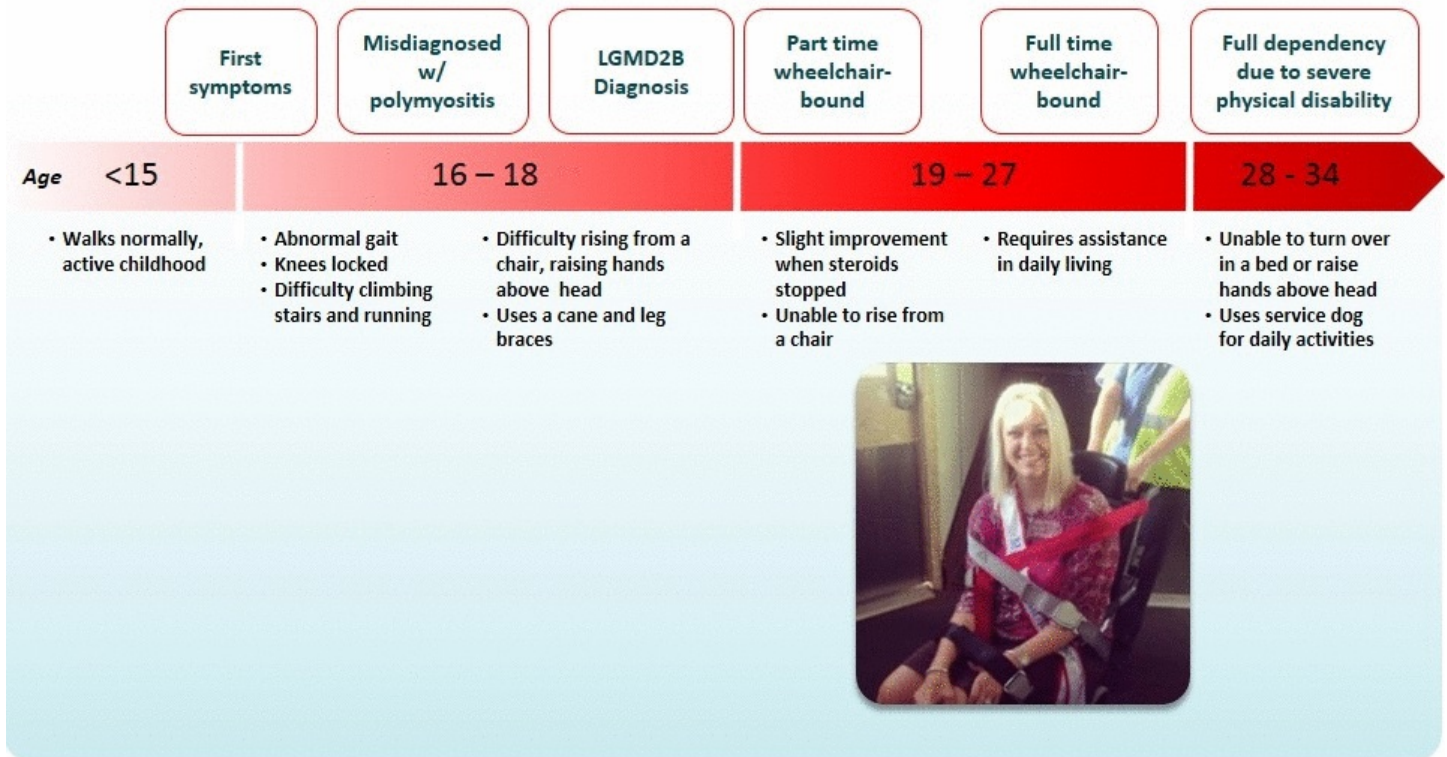
Trial	Indication(s)	Patients	Highest Dose	Design
002	Adult FSHD	3 dose cohorts (n=20 Total)	3.0 mg/kg Weekly (12 weeks)	Placebo controlled, Double blinded; Interpatient Dose Escalation up to 12 weeks
003	Early onset FSHD	(n=8)	3.0 mg/kg Weekly (6 weeks)	Open-label, Inpatient Dose Escalation for 12 weeks
004	Adult LGMD2B, Adult FSHD	LGMD2B (n=10) FSHD (n=8)	3.0 mg/kg Biweekly (4 weeks)	Open-label, Inpatient Dose Escalation for 12 weeks

*MMT = Manual Muscle Testing, a validated assessment tool that measures muscle function/strength

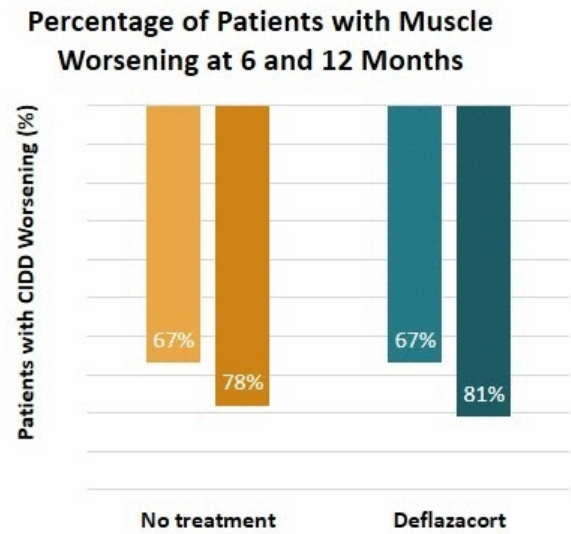
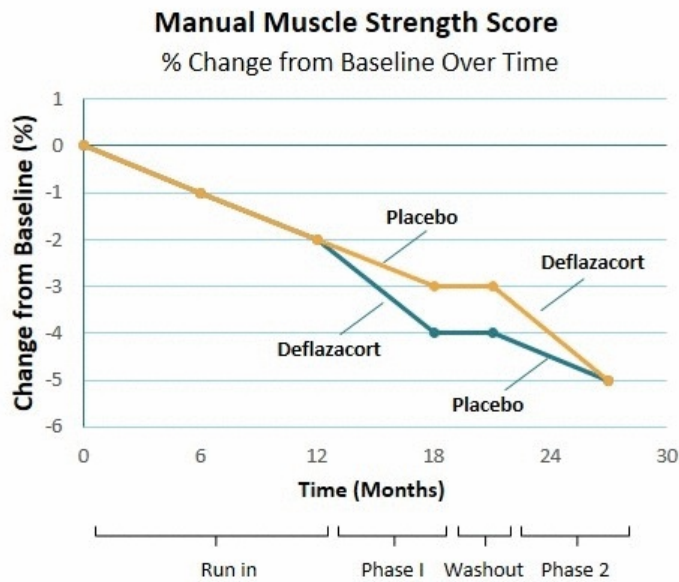
INQoL = Individualized Neuromuscular Quality of Life, a patient reported outcome measure designed specifically for neuromuscular disease

LGMD2B Disease Progression Case History

PATIENT
CASE HISTORY



<https://www.youtube.com/watch?v=JLaHis1vPUI>
<http://mwtn2013blisswelch.blogspot.com/>



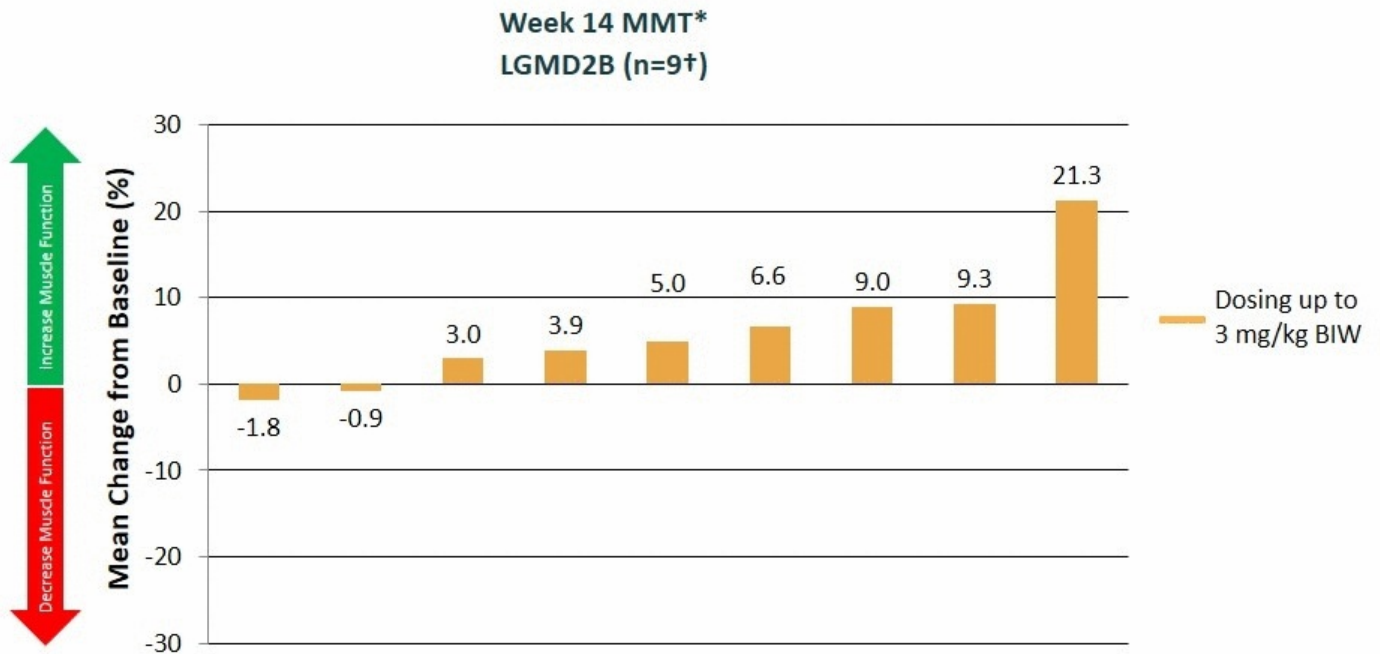
Treatment with Deflazacort was for 6 months in each arm. Single site, placebo controlled, cross over design (n=25)

Manual muscle strength assessed bilaterally by the modified Medical Research Council Scales (MRC)
CIDD (Clinical Investigation of Duchenne Dystrophy) score, graded from 0 (worst) to 10 (best)

Manual Muscle Test (MMT) Scores LGMD2B Patients

004 Study: Individual Patient Changes from Baseline (%)

RESOLARIS
PROGRAM



*1-week follow-up is earlier than week 14 for 2 early discontinuations; Manual Muscle Testing (MMT) a validated assessment of muscle function/strength in 14 muscle groups

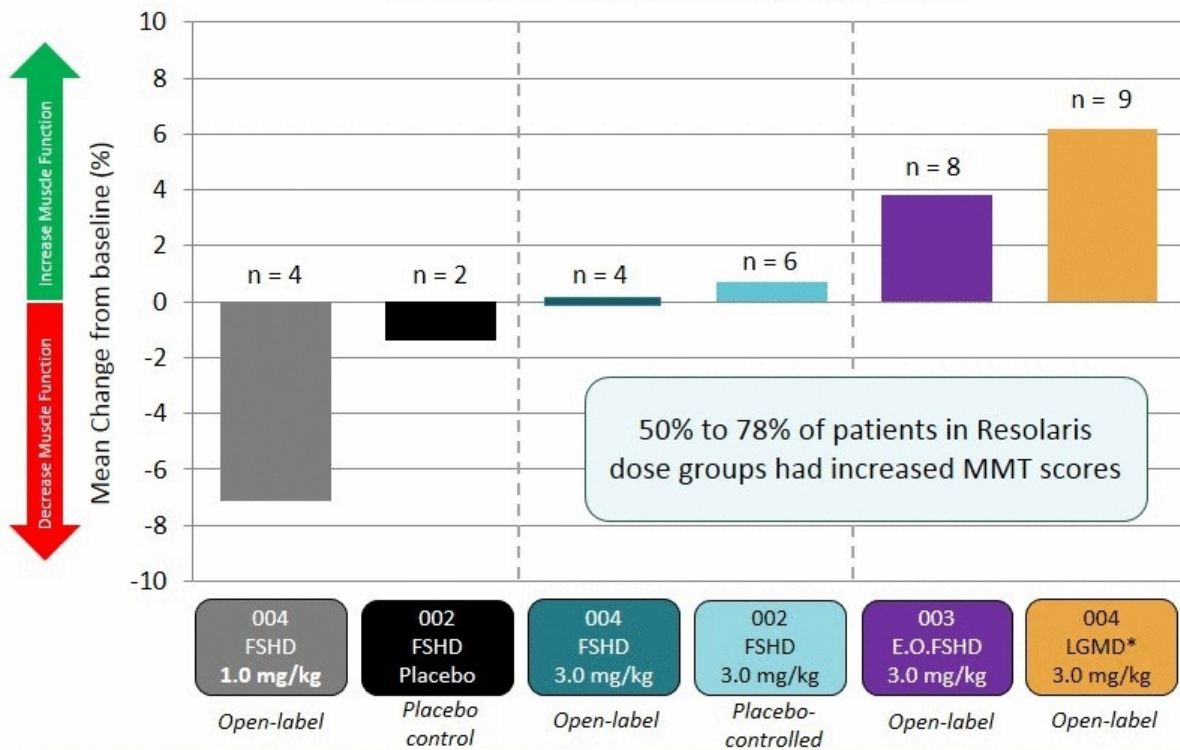
† One patient in 004 Trial did not have an MMT measurement due to being wheelchair bound at baseline

Compiled Data from Three Phase 1b/2 Clinical Trials

Relatively Stable or Improved Muscle Function Observed

RESOLARIS
PROGRAM

Overall Mean MMT* Change Week 14 by Dose Group
FSHD & LGMD2B Patients From 002, 003, 004 Trials



* Manual Muscle Testing (MMT) a validated assessment of muscle function/strength in 14 muscle groups

No observed signs of general immunosuppression

Consistent with a homeostatic pathway working at a tissue level

Well-tolerated across all doses tested:

Multiple myopathies; various age-groups; long-term exposure
No serious adverse events reported by investigators

Low-level anti-drug antibody assay signals did not result in clinical symptoms
Protocol discontinuations primarily driven by transient infusion related reactions

Target Product Profile (Discontinuation Rate \leq 10%)

- *Potential to pre-medicate patients*
- *Potentially relax cut-off criteria for discontinuations*



STUDY 003

EARLY ONSET FSHD



Early Onset FSHD Case History

Early progression, devastating disease impact

CLINICAL
DEVELOPMENT



Age	<6	6 – 12	12 – 18	18 - 24	
	<ul style="list-style-type: none"> • Normal early childhood 	<ul style="list-style-type: none"> • Muscle weakness • Early speech impediment • Difficulty forming facial expressions 	<ul style="list-style-type: none"> • Weakness in lower back/curvature of the spine • Leg muscle weakness/ walking with a limp 	<ul style="list-style-type: none"> • Lower limb muscle weakness/walker or chair • Development of severe speech impediment 	<ul style="list-style-type: none"> • Foot drop and loss of ability to stand/multiple falls
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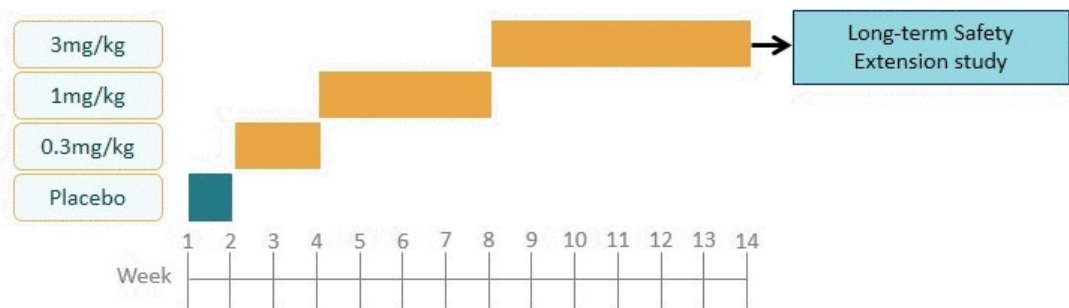
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Climbing Mountains; Sarabjit Parmar, 2014

003 Study Design

Early onset FSHD

STUDY 003

- **Purpose:** to evaluate the safety/tolerability of Resolaris in adolescents and young adults with early onset FSHD
- **Design:** multicenter, open label, intra-patient dose escalation
- **Sites:** 5 sites in the US, France, and Italy
- **Population:** genetically confirmed FSHD and signs/symptoms of FSHD by age 10 years
 - Stage 1 – 16-25 years old*

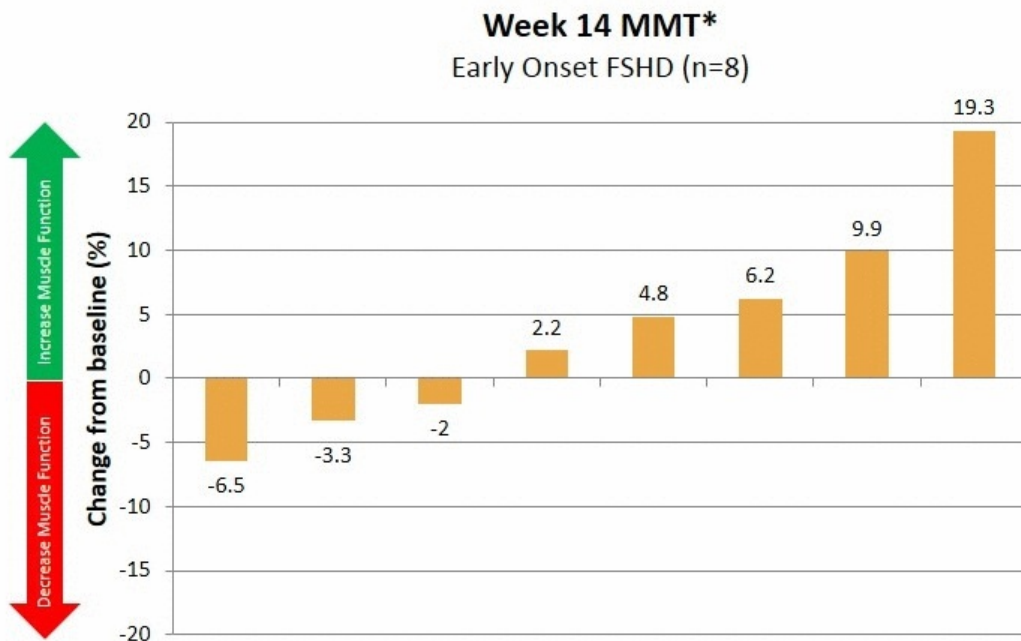


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MMT Scores Early Onset FSHD Patients

Individual Patient Changes from Baseline (%)

STUDY 003

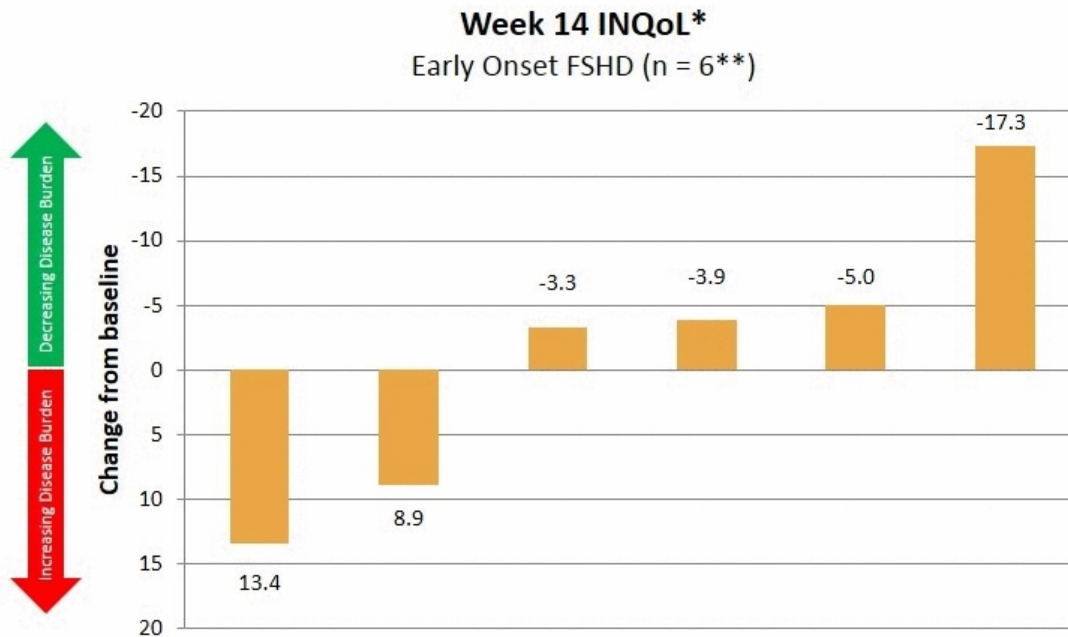


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INQoL Scores Early Onset FSHD Patients

Individual Patient Changes from Baseline

STUDY 003



* Individualized Neuromuscular Quality of Life (INQoL) score is a validated patient reported outcome measuring a patient's level of disease burden

** Excludes two patients due to (1) no baseline and (2) discontinuation before week 14

Safety

- Resolaris was generally well tolerated in patients ages 16 to 20 years in the study
- Adverse Events (AEs) were mild to moderate in severity and consistent with overall Resolaris safety profile
- No Serious Adverse Events
- No observed signs of general immunosuppression
- One patient (03-001) discontinued due to an infusion-related reaction
- No patients discontinued due to Jo-1 criteria (≥ 1.5)
- No dose limiting changes in lab parameters, vital signs or pulmonary tests

Other Assessments

- Surveillance MRI: little change in STIR positivity or Fischer Scores
- Ophthalmology and Hearing Assessments: little change in any of the parameters assessed
- Biomarker analysis: levels mostly low-to-normal (48/52); no consistent trends

Milestones

- ✓ Muscle Function Signals: Adult LGMD2B; Early onset FSHD > Adult FSHD
- ✓ Established a favorable safety profile and identified an active dose
- ✓ Commercial scale manufacturing to be ready for future larger randomized controlled trials
- ✓ Fast Track designations for Resolaris to treat FSHD and LGMD2B

2017 Development Goals

First Half

- ✓ **Clinical Results:** Early Onset FSHD Patient Trial (003)

Biomarker/MOA: Introduce Mechanistic/PD Assay

Second Half

Clinical Trial: Kick off next trial post partnership*

*Partner for one or more programs

A photograph of green leaves reflected in water, with text overlaid. The leaves are in the foreground, and their reflection is visible in the water below. The background is a soft, out-of-focus light blue and green.

BUILDING A NEW CLASS OF THERAPEUTICS FOR PATIENTS
FOUNDATION FOR THE FUTURE

LIFE Leaders

FOUNDATION
FOR THE FUTURE

	<div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>John Mendlein, Ph.D. Chief Executive Officer</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Sanuj Ravindran, M.D. Chief Business Officer</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Sanjay Shukla, M.D. Chief Medical Officer</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>David King, Ph.D. SVP, Research</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Grove Matsuoka SVP, Product Programs and Planning</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>John Blake, CPA SVP, Finance</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Andrea Cubitt, Ph.D. VP, Product Protection</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Ashraf Amanullah, Ph.D. VP, Manufacturing</p> </div> <div style="border: 1px solid black; padding: 5px; margin-bottom: 5px;">  <p>Holly D. Chrzanowski VP, Enterprise Talent and Organization</p> </div> <div style="border: 1px solid black; padding: 5px;">  <p>Nancy Krueger VP, Legal Affairs</p> </div>	
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2017 Goals

- Partner One or More Programs
- Advance Pipeline with Two Molecules in the Clinic
- Declare 3rd IND Candidate from Physiocrine Discovery Engine

Financial Guidance

- \$76M cash 2016 EOY
- Operations funded into 3Q 2018 without any partnerships
- ~30% expected reduction in operational cash burn compared to 2016*

**Operational cash burn only, excludes cash from financings*



GRACIAS

MERCI

多謝

СПАСИБО

متشكراً

תודה.

SUKRIA

ありがとう

谢谢

EFHARISTO

DANKE

SHUKRAN

KHOP KHUN MAK KHA

MAHALO

OBRIGADO

GRAZIE

SPASIBA

DZIEKUJE

감사합니다

THANK YOU!

TAKK

TERIMA KASIH

NANDRI



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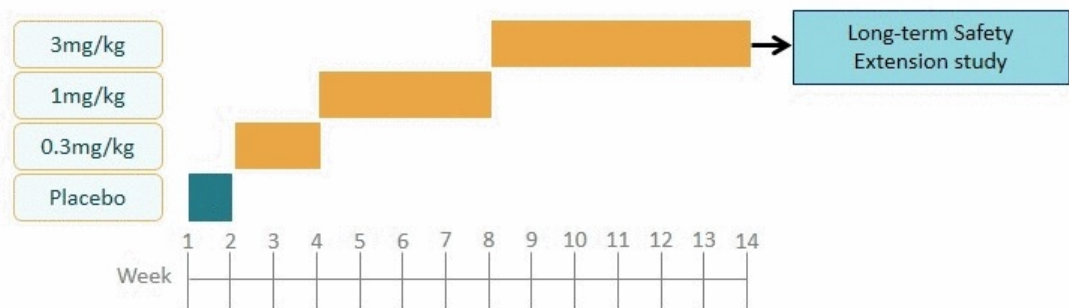
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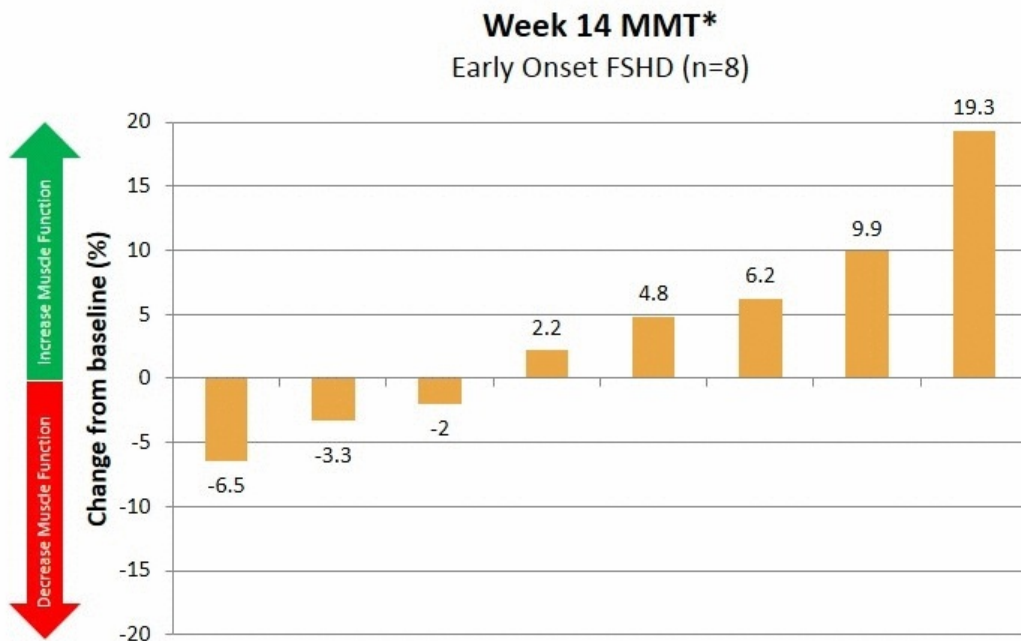


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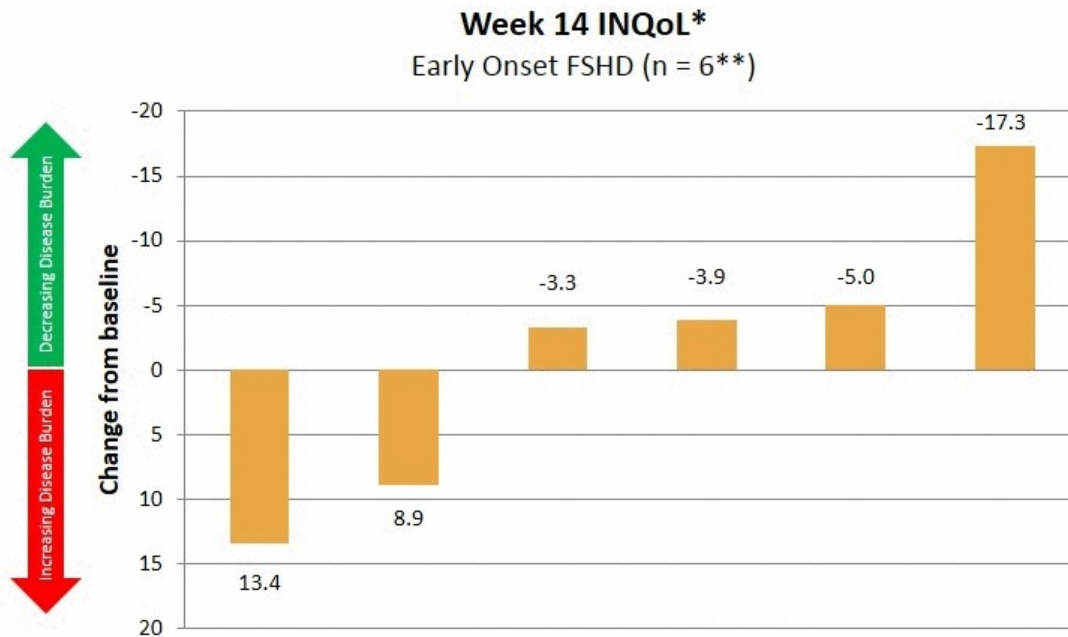


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