

**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

Date of Report (Date of earliest event reported): April 13, 2016

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
(I.R.S. Employer
Identification No.)

**3545 John Hopkins Court, Suite #250
San Diego, CA 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 obligation 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))
-
-

Item 7.01 Regulation FD Disclosure.

aTyr Pharma, Inc. (the “Company”) intends to use an investor presentation to conduct meetings with investors, stockholders and analysts and at investor conferences, and which the Company intends to place on its website. A copy of the presentation materials is attached hereto as Exhibit 99.1. The Company does not undertake to update the presentation materials.

The information under this Item 7.01, including Exhibit 99.1, 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 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Corporate Presentation Materials of aTyr Pharma, Inc. dated April 2016

SIGNATURES

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.

Date: April 13, 2016

aTyr Pharma, Inc.

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

EXHIBIT INDEX

Exhibit No.

Description

99.1

Corporate Presentation Materials of aTyr Pharma, Inc. dated April 2016



*Building a New Class of Medicines
- Physiocrine Based Therapeutics*

*1st Rare Disease Trial Completed
- New Hope for Muscular Dystrophy Patients*

April 2016



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 iMod. Fc, 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, 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 most recent Annual Report on Form 10-K. 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 and Resolaris™. 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.



Augmenting Natural Homeostatic Pathways in Patients with Rare Diseases of Muscle & Lung

ATYR
HIGHLIGHTS

Physiocrine*
Disruptive
Opportunity

Pioneering new biology, yielding
new therapeutic intervention points

Focusing on natural modulators of
immune & fibrotic pathways

Resokine:
One Pathway
Many Rare Diseases

Connecting Immune/fibrotic nexus to
rare muscle & lung diseases

Dose above normal levels to therapeutically
promote tissue homeostasis & restoration

Resolaris
Encouraging
Phase 1b/2 Data

1st muscular dystrophy trial completed
3 ongoing trials in muscular dystrophies

1st safety & tolerability data in patients
1st potential activity signal in FSHD

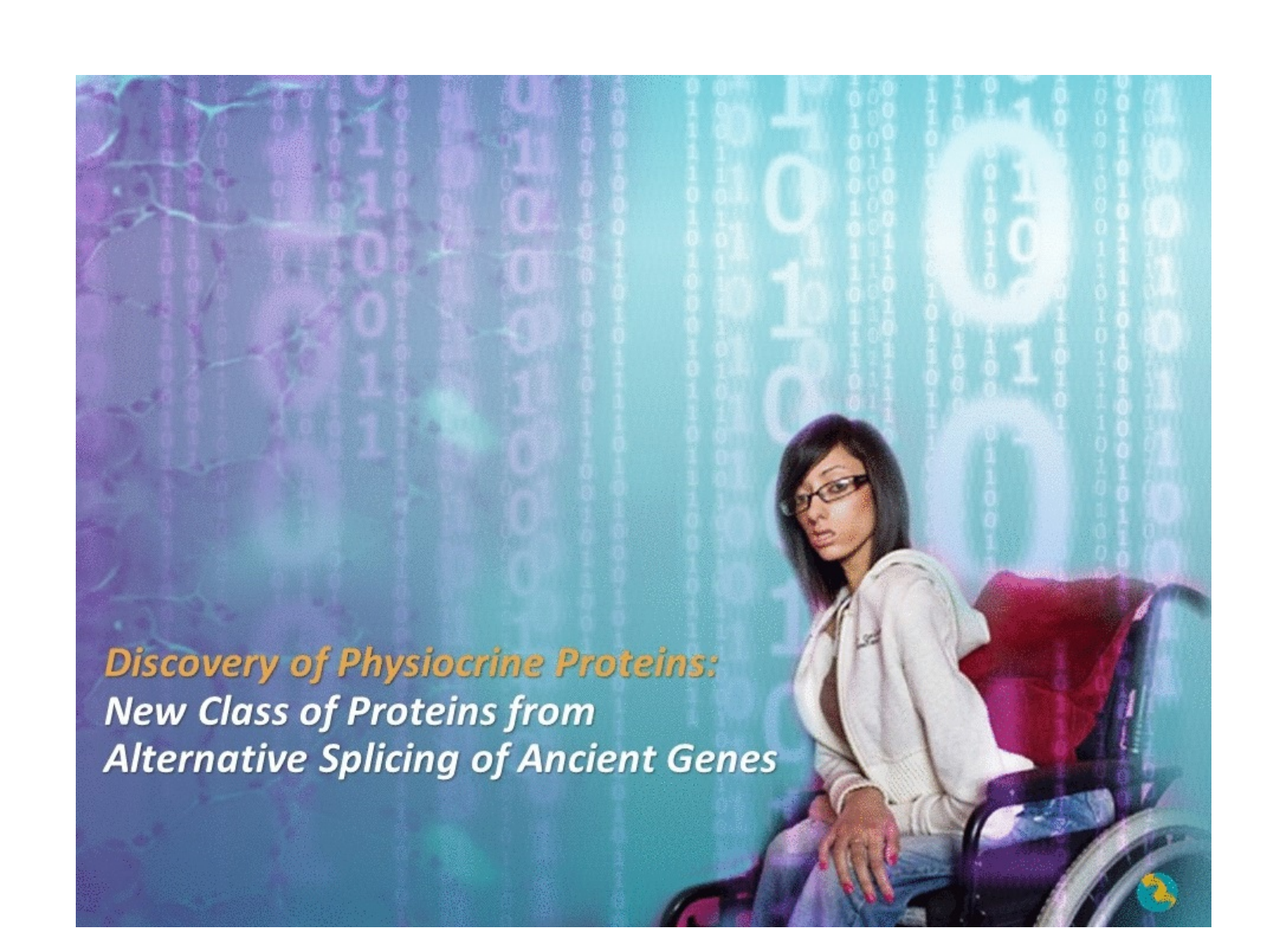
Autonomous
Pipeline &
Business Model

1st in class biologics pipeline
2nd Program iMod.Fc for lung disease

Unique investment thesis: build franchises in
muscle, lung & liver based on new biology

*Proteins for life (physio) specific activity (crine)



A woman with dark hair and glasses, wearing a white hoodie and blue jeans, is seated in a wheelchair. She is looking towards the camera. The background is a digital-themed image with vertical columns of binary code (0s and 1s) in shades of blue and green. On the left side, there is a faint, glowing molecular or cellular structure. The overall lighting is soft and futuristic.

***Discovery of Physiocrine Proteins:
New Class of Proteins from
Alternative Splicing of Ancient Genes***



aTyr Pioneering the New Biology of Physiocrines

THERAPEUTICALLY
TAPPING
NOVEL PATHWAYS

Science 1999

Nature 2010

Nature 2013

Science 2014

Nature 2015

~300 proteins involved in physiological pathways, >70 issued patents

Focus on natural modulators of immune & fibrotic pathways in vivo

Indication selection: preclinical & clinical phenotype overlap

Resolaris focused on rare myopathies with an immune component

iMod.Fc focused on rare lung diseases with an immune & fibrotic component

aTyr Pharma

Properties of Physiocrines

Potential for novel physiological modulation in disease

THERAPEUTIC
POTENTIAL

At least 14/23 tRNA Synthetase family with known disease connections

Clinical phenotypes suggest potential for tissue-by-tissue homeostasis products

Extracellular functions of 4 billion year old gene family, tRNA Synthetases

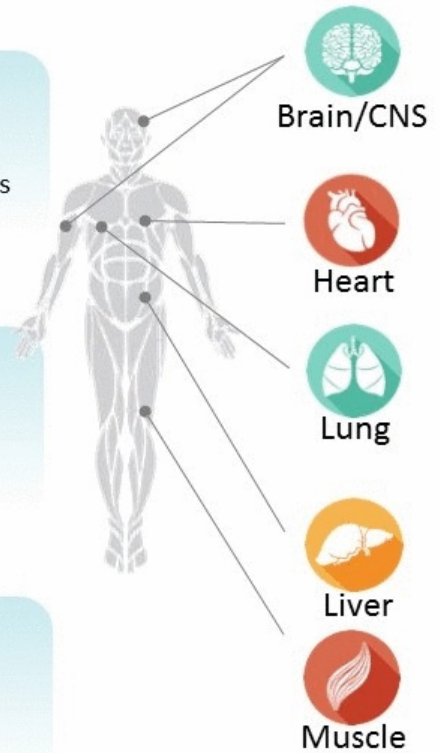
Genes yield ~300 proteins (e.g. alternative splicing, etc.) of new function

Potential new class of modulators of tissue homeostasis

Work via GPCRs, TLRs, cytokine receptors & other proteins

Not glycosylated & non-canonical leader sequences

Size range 40-500AA



Leadership Team

EXPERIENCED
INDUSTRY VETERANS



John Mendlein, Ph.D.
Chief Executive Officer



Sanuj Ravindran, M.D.
Chief Business Officer



Sanjay Shukla, M.D.
Chief Medical Officer



Andrew Cubitt, Ph.D.
VP, Product Protection and
Interim Head of Research



Kelly Blackburn
VP, Clinical Operations



Ashraf Amanullah, Ph.D.
VP, Manufacturing



John Blake, CPA
VP, Finance



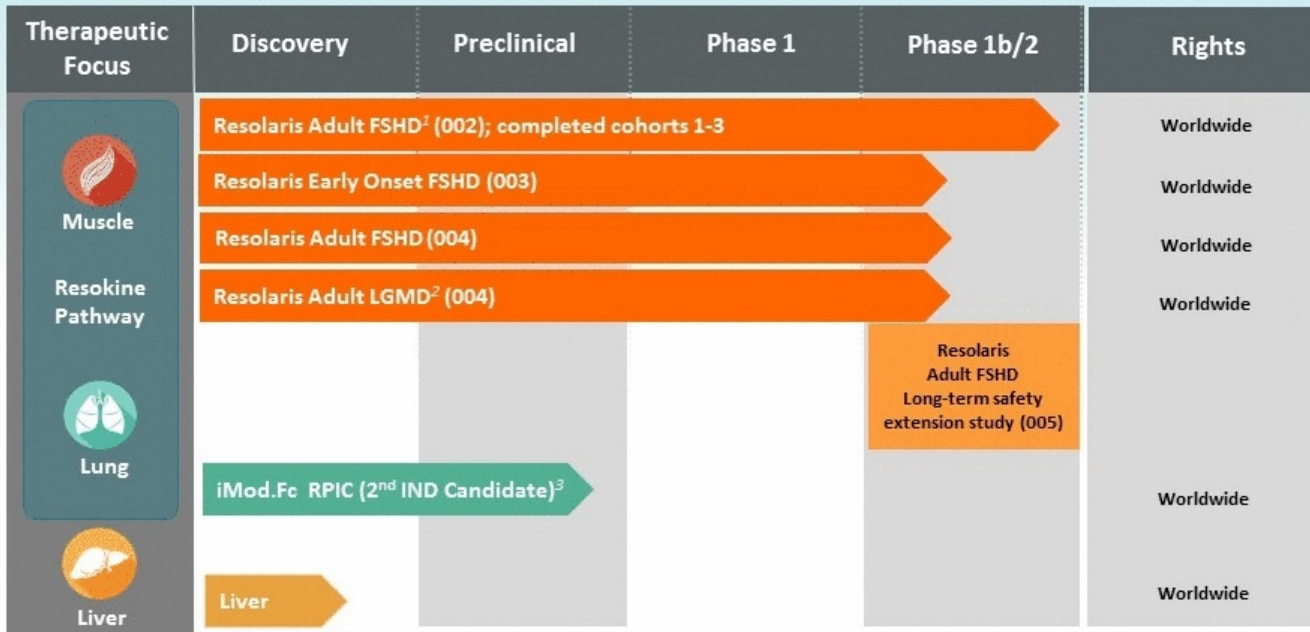
Holly D. Chrzanowski
VP, Enterprise Talent &
Organization



Immuno/Fibrosis Modulation Pipeline

1st in class candidates for rare diseases with an immune or fibrotic component

PARADIGM SHIFT IN TREATMENTS



Patient Phenotype Focus
 Severe impact from disease with the potential for large treatment effect
 Subject to poor standard of care

¹ Facioscapulohumeral Muscular Dystrophy

² Limb-girdle Muscular Dystrophy

³ RPIC: Rare pulmonopathies with an immune component, including Interstitial Lung Disease



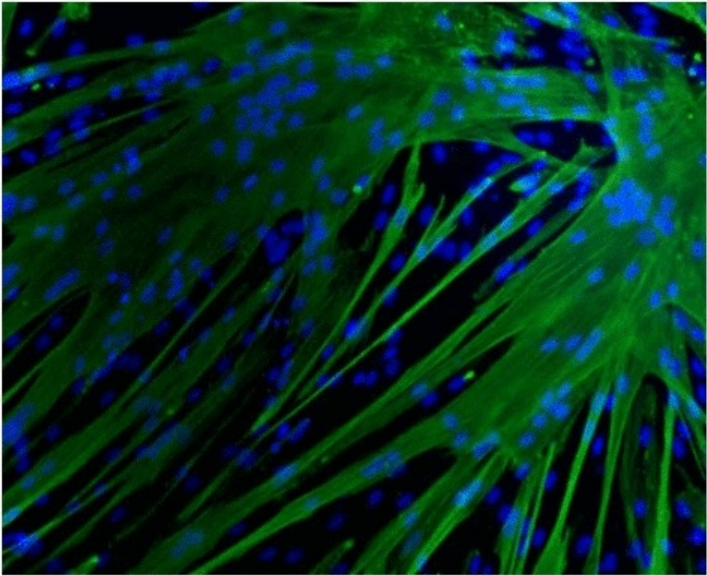
Harnessing the Resokine* Pathway:

***Potential New Therapies for Patients
with Rare Muscle & Lung Diseases***

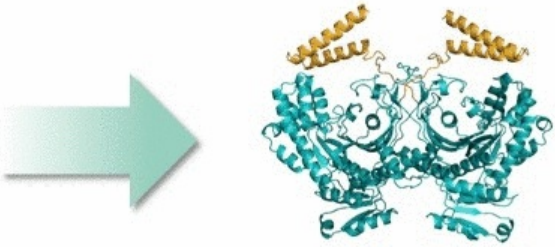
**Resokine: for resolution of activated immune & fibrotic pathways*



Resokine Released From Human Primary Skeletal Muscle Cells



IGF1 Facilitated Differentiation
Human Skeletal Muscle



Resokine Released from
Human Skeletal Muscle



Molecules of the Resokine Pathway

New hope for rare muscle & lung disease patients



RESOKINE
FRANCHISE

Resokine* Pathway Discovery:

- Clinical correlates: myopathies & lung diseases where we believe the pathway is disrupted
- Immuno-modulatory & fibro-modulatory activity via in vivo screening



Therapeutic applications in patients with similar phenotype:

- Rare myopathies with an immune component (RMICs) 
- Rare pulmonopathies with an immune component (RPICs) 

*Resokine (name for extracellular HARS, a tRNA Histidyl Synthetase)

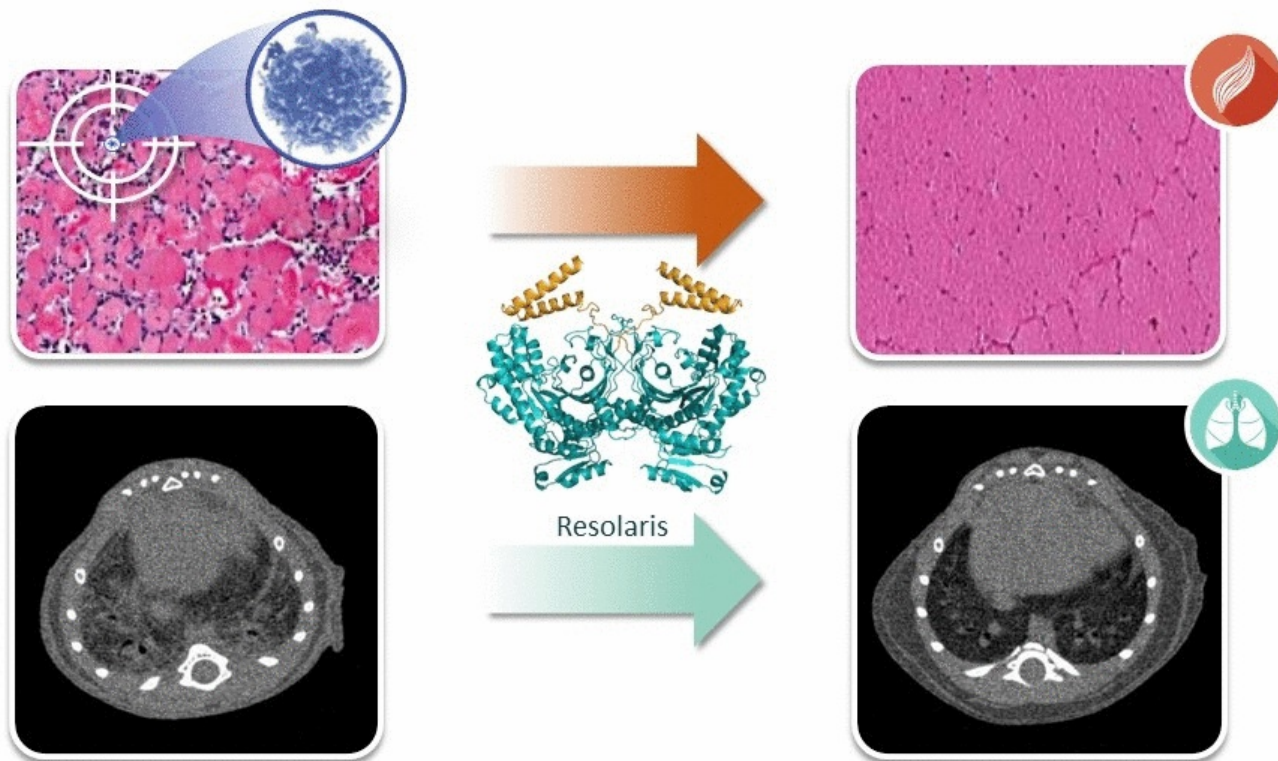
**Zhou et al, JBC, 2014



Resolaris Improves Muscle & Lung Immune/Fibrotic Components

AUGMENTING
THE RESOKINE
PATHWAY

Two weeks of therapeutic treatment in Statin myopathy & Bleomycin IPF* models



*Idiopathic Pulmonary Fibrosis ("IPF"), a subset of Interstitial Lung Disease ("ILD")



Clinical Path for Resolaris & iMod.Fc

Staging Rare Muscle & Lung Disease Indications

DEVELOPING A NEW CLASS OF PRODUCTS

1st Physiocrine based therapeutics to promote homeostasis

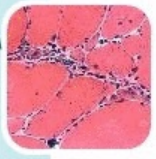
Resolaris



Adult FSHD 1b/2 Trial Completed



Early Onset FSHD 1b/2 underway



Adult Limb Girdle Muscular Dystrophy 1b/2 underway

Exploratory stage trials in rare diseases

iMod.Fc



Severe Rare Lung Diseases

On track for 2017 clinical trial

Establish & explore:

1. Safety, tolerability
2. Activity/end-point potential

Multiple opportunities for advancement

For patients with few or no treatment options





***Clinical Development of Resolaris:
Harnessing a Natural Pathway
to Treat Multiple Rare Muscle Diseases***

FSHD: A Severe Skeletal Muscle Disease

With dominant/spontaneous genetic toxic gain of function

DISEASE
PRESENTATION

Facioscapulohumeral Muscular Dystrophy (FSHD)

Pathology

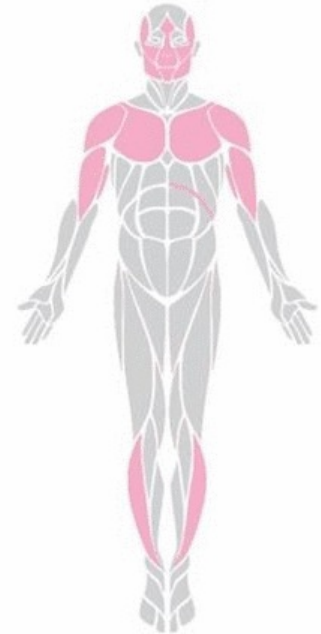
- Dominant/spontaneous toxic gain of function (Dux4)
- Immune component (e.g. T cells)
- Muscle loss, fat infiltration

Clinical

- Debilitating muscle weakness
- Difficulty raising arms, foot drop when walking
- Mouth atrophy; difficulty smiling and swallowing
- Slow progression of disease systemically
- May have visual or auditory impairment

Standard of care

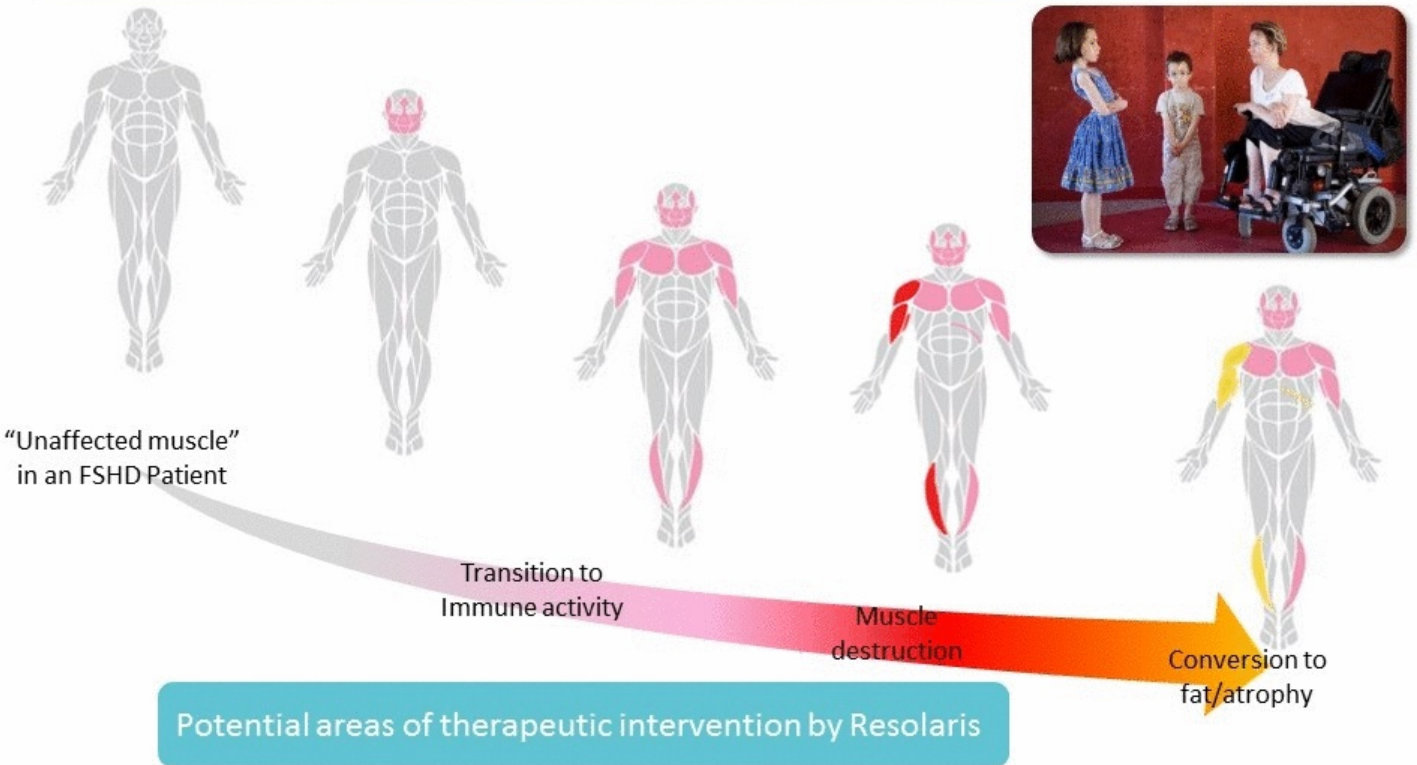
- No therapeutic treatments
- Only supportive care provided



Slow Muscle-by-Muscle Disease Progression

Therapeutically promoting homeostasis in FSHD Muscle throughout the body

INTERVENING
EARLY



First Physiocrine Patient Trial Data

Building a foundation for a franchise and new class of therapeutics

1ST FSHD TRIAL
HIGHLIGHTS

Significant step forward to advancing Physiocrine based therapeutics

Important advancement for hard to treat diseases (FSHD/RMICS)

- Establishing data dossier on safety
- Exploring activity assessments & optimal dose
- Directionality on endpoints for approval

Foundation to build upon with 4Q 2016 trial data



Design

Double-blinded, 4 sites, 4 countries, n=20,
Multiple Ascending Dose, 3:1 Randomization (Resolaris:placebo)

- | | |
|--|--|
| <p>1. Build dossier for Resolaris & class:</p> <ul style="list-style-type: none">• Safety• Tolerability• Immunogenicity• PK | <p>✓ Completed 1st multiple dose trial</p> <ul style="list-style-type: none">✓ Safety✓ Tolerability*✓ Immunogenicity✓ PK |
| <p>2. Explore FSHD pertinent readouts</p> <ul style="list-style-type: none">• Circulating markers of disease• Targeted MRI of disease muscle• Strength• Patient reported outcomes | <p>✓ Completed 1st FSHD only trial</p> <ul style="list-style-type: none">± Only 2/20 patients with elevated levels± Targeted may be too narrow✓ Muscle testing may hold promise✓ Potential signal to confirm |
| <p>3. Across 3 dose cohorts over 1 or 3 months of dosing</p> | <p>✓ Potential activity @ 3.0 mg/kg (weekly)</p> |

* 1 reversible Infusion Related Reaction (IRR) patient in 002 & 2 reversible IRR patients in 005 trial



Exploratory Study of Resolaris in Adult FSHD

Baseline Study Characteristics

RESOLARIS
CLINICAL

Study Demographics

	Resolaris	Placebo
Patients	15	5
Age (years), Median	46	52
Male/Female (%)	53/47	80/20
Patients with 3 D4Z4 repeats or less	2	0
Completed Study	100%*	100%
Elevated cytokines of interest	1/15	1/5
Baseline FSHD Clinical Severity Score, Mean (SD)	3.2 (0.8)	2.7 (0.7)

Analysis based on data available through early March 2016

* One patient discontinued dosing at week 11 of the 12 weeks of treatment, but completed all study visits.



Establishing the Safety Profile of Resolaris

Most adverse events in treatment arm were mild to moderate

1ST FSHD TRIAL

RESOLARIS

SAFETY

Generally safe and well-tolerated over the dose range/duration studied

No SAEs reported by study investigators

- One generalized infusion related reaction (IRR) reclassified by aTyr to serious adverse event

ADAs confirmed in approximately 40% of patients dosed with Resolaris

- No demonstrated effect of ADAs on pharmacokinetics of Resolaris and of low titer
- No reported antisynthetase syndrome

Procedures in effect to minimize occurrence of IRRs/ADAs moving forward



INQoL: Patient Reported Outcome

Individual Neuromuscular Quality of Life Assessment

CLINICAL
READOUTS

Validated neuromuscular assessment tool*

- Global systemic assessment used in clinical studies and trials
- Not frequently used in clinical practice

Self-administered questionnaire consisting of 45 queries/4 dimensions

1. Life Domains
 - 1) Activities 2) Independence 3) Social Relationships 4) Emotions 5) Body Image
2. Symptoms, Treatment Effects
3. Overall INQoL score; derived from 5 Life Domains

Improvement = decreased scores

- In individual Life Domains & Overall INQoL (negative change from baseline)

FDA: "Generally, findings measured by a well-defined and reliable PRO instrument in appropriately designed investigations can be used to support a claim in medical product labeling if the claim is consistent with the instrument's documented measurement capability."**

* Vincent KA et al: Construction and Validation of a Quality of Life Questionnaire for Neuromuscular Disease (INQoL). Neurology 2007, 68:1051-1057.

** FDA Guidance for industry. Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims; 2009.



Multiple Dimensions Improve on INQoL

Improvement reflected by negative change from baseline

Treatment Duration Group	INQoL Overall Scores Change from Baseline (%) ITT Population (n=20)			
	Placebo	0.3 mg/kg	1.0 mg/kg	3.0 mg/kg
1 Month	4.12 (n=5)	2.77 (n=3)	-1.22 (n=4)	-3.78 (n=6)
3 Months	15.55 (n=2)	NA ¹	NA ¹	-9.90 (n=6)

Overall Score: five Life domains:

- Activities
- Independence
- Emotions
- Social Relationships
- Body Image

Treatment Duration Group	Proportion of Patients with Improved INQoL Overall Scores			
	Placebo	0.3 mg/kg	1.0 mg/kg	3.0 mg/kg
1 Month	2/5	1/3	2/4	4/6
3 Months	0/2	NA ¹	NA ¹	5/6

Trial not powered to show statistical significance

Data suggest potential improvement in this relatively small clinical trial of FSHD patients

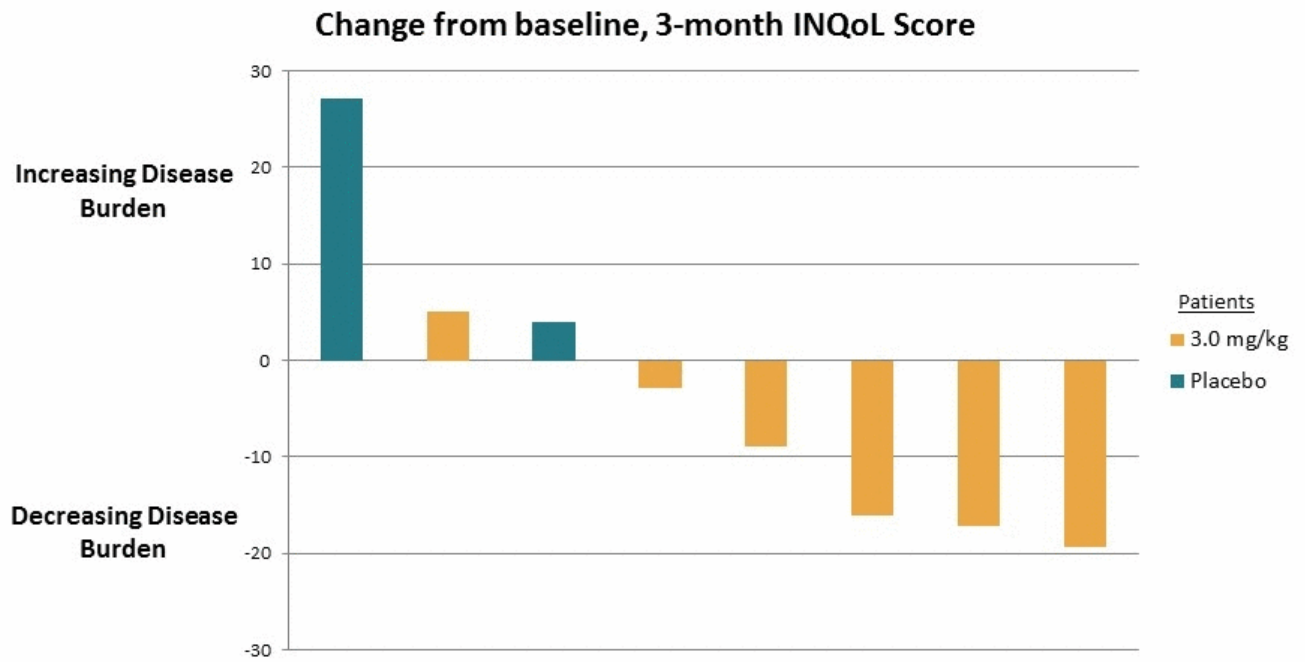
1) NA is not applicable; only 1 month of dosing

2) Relative improvement placebo v. 3.0 mg/kg cohort at 3 months: 25.5% (p=0.03)



Placebo v 3.0 mg/kg Patients Overall INQoL Score

Suggestive of patient improvement in 3 months



MMT Measurement

Muscle function/strength was formally assessed by investigators using Manual Muscle Testing (MMT)

- 15 muscles evaluated at 4 time points in study
- Muscles scored individually
- Composite score calculated
- Progression: lower scores; negative change from baseline
- Improvement: higher scores; positive change from baseline



Data suggest small trends of slower progression or potential improvement



Targeted MRI & Circulating Markers

To be monitored in additional studies and extension phase

1ST FSHD TRIAL

RESULTS

ACTIVITY

MRI used to evaluate immune components in a targeted muscle did not record a difference between placebo and 3.0 mg/kg group

- To be followed through extension study

No evidence of immune suppression was observed with exploratory circulating cytokines, as well as immune cells

Assessment of selected circulating markers did not record a difference between placebo and 3.0 mg/kg group

Only 2 subjects started with elevated levels of immune markers of interest



LGMD: A Severe Muscle Disease

With a genetic loss of function

DISEASE
PRESENTATION

Limb-girdle Muscular Dystrophy 2B (LGMD2B)

Pathology

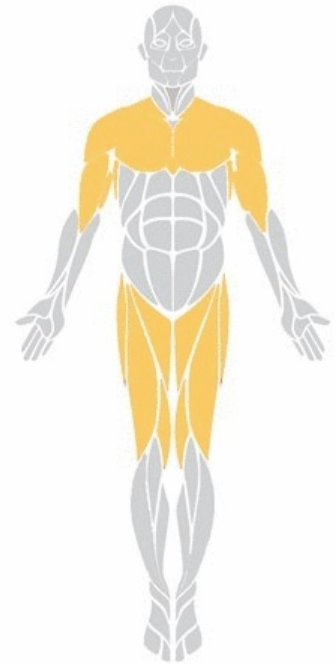
- Immune component (e.g. T-cells)
- Toxic loss of function mutation (dysferlin)
- Muscle group progression

Clinical

- Debilitating muscle weakness
- Challenges moving limbs
- May have respiratory insufficiency

Standard of care

- No therapeutic treatments
- Only supportive care provided



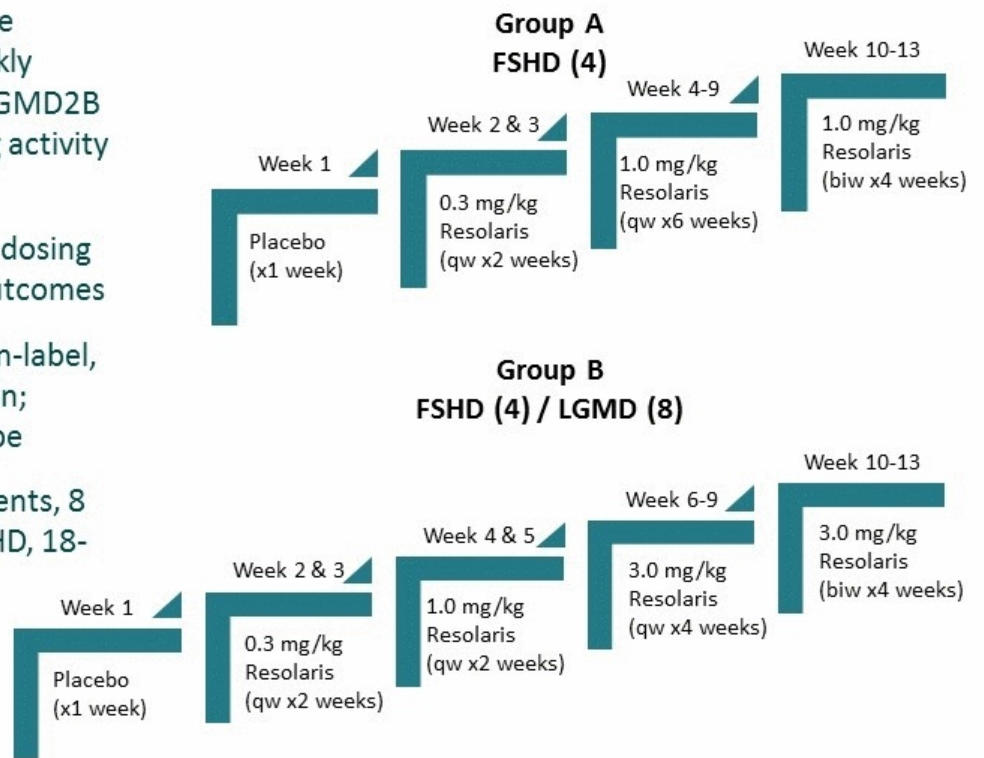
Adult LGMD2B and FSHD (Trial-004)

Purpose – 1) to evaluate the safety/tolerability of biweekly infusions in patients with LGMD2B and FSHD; 2) to assess drug activity more proximal to dosing

Rationale – More frequent dosing may demonstrate better outcomes

Design & study sites – Open-label, intra-patient dose escalation; multiple sites in US & Europe

Study population – 16 patients, 8 each with LGMD2B and FSHD, 18-75 years of age



Early Onset FSHD Case History

Early progression, devastating disease impact

PATIENT
STORY

First symptoms

Diagnosed age 8 with FSHD

Diagnosed with scoliosis

Part time wheelchair-bound

Full time wheelchair-bound

Full dependency due to severe physical disability

Age <6

- Normal early childhood

6 – 12

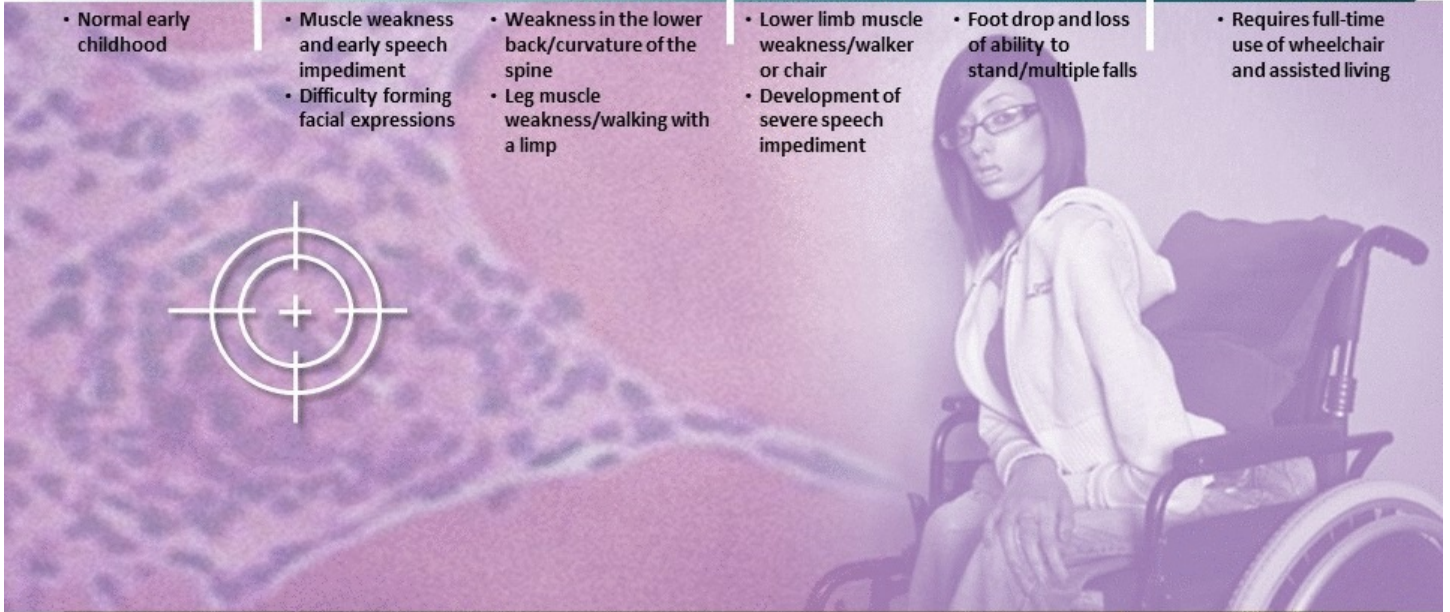
- Muscle weakness and early speech impediment
- Difficulty forming facial expressions
- Weakness in the lower back/curvature of the spine
- Leg muscle weakness/walking with a limp

12 – 18

- Lower limb muscle weakness/walker or chair
- Development of severe speech impediment
- Foot drop and loss of ability to stand/multiple falls

18 - 24

- Requires full-time use of wheelchair and assisted living



<http://www.theguardian.com/lifeandstyle/2009/may/28/muscular-dystrophy-disability-fshd>
Climbing Mountains; Sarabjit Parmar, 2014



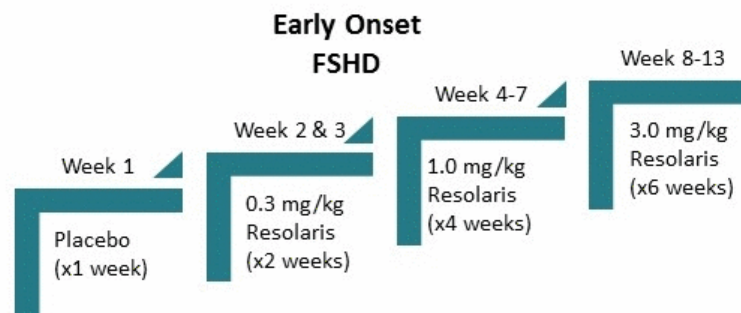
Early Onset FSHD (Trial-003)

Purpose – 1) to evaluate the safety/tolerability of weekly infusions in patients with Early Onset FSHD; 2) to assess drug activity in new patient population & with additional endpoints

Rationale – Investigate often more severe form of disease, involves additional organ systems

Design & study sites – Open-label, intra-patient dose escalation; multiple sites in US & Europe

Study population – 16 patients; Stage 1: 8 patients 16-25 years of age; Stage 2: 8 patients 12-15 years of age. Genetically confirmed diagnosis of FSHD and onset of symptoms prior to age 10.



Resolaris Trial Summary 2016

Trial	Patient Populations	N	Highest Ending Dose Weekly (mg/kg)	MRI (+) Entrance	MRI Broad Readout	MRI Targeted Readout	INQoL	MMT	Immune Markers	Readout Timing
002	Adult FSHD	20	3.0	✓	Yes	✓	✓	✓	✓	✓
003	EO FSHD	8	3.0	No	Yes	No	Yes	Yes	Yes	4Q16
004	Adult FSHD	8	3.0 (2x)	Yes	Yes	Yes	Yes	Yes	Yes	4Q16
004	Adult LGMD	8	3.0 (2x)	Serum marker or MRI	Yes	Yes*	Yes	Yes	Yes	4Q16
005	Adult FSHD (002 ext. study)	8	3.0	No	Yes	Yes**	Yes	Yes	Yes	4Q16

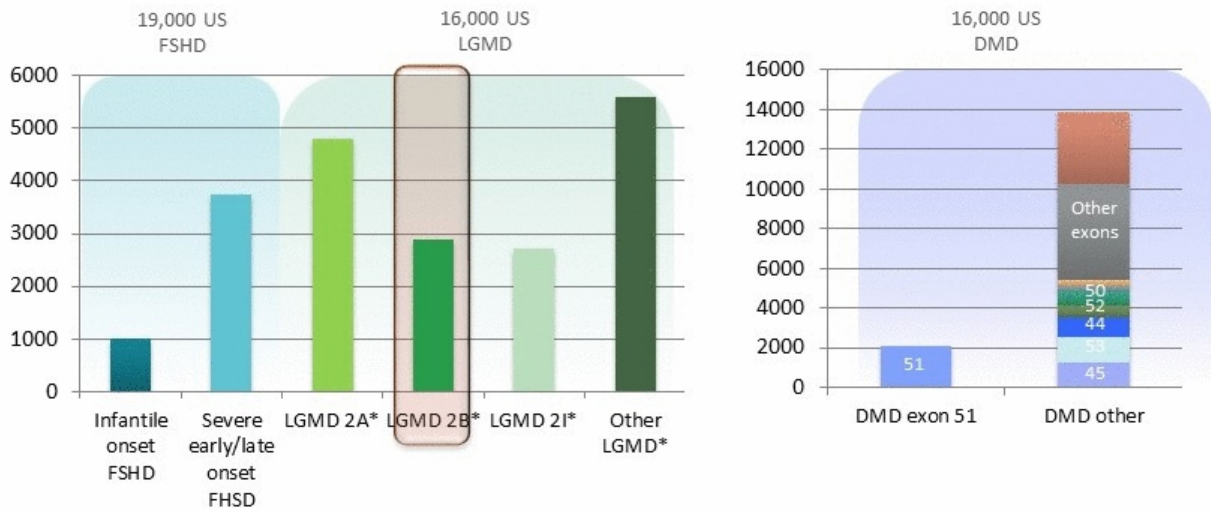
Next FSHD trial based on 4Q results of 003, 004 & 005 trials

* Targeted MRI only if qualified with Stir+ muscle at baseline.
 ** Only for Cohort 3 subjects

Resolaris: One Product, Multiple Rare Diseases

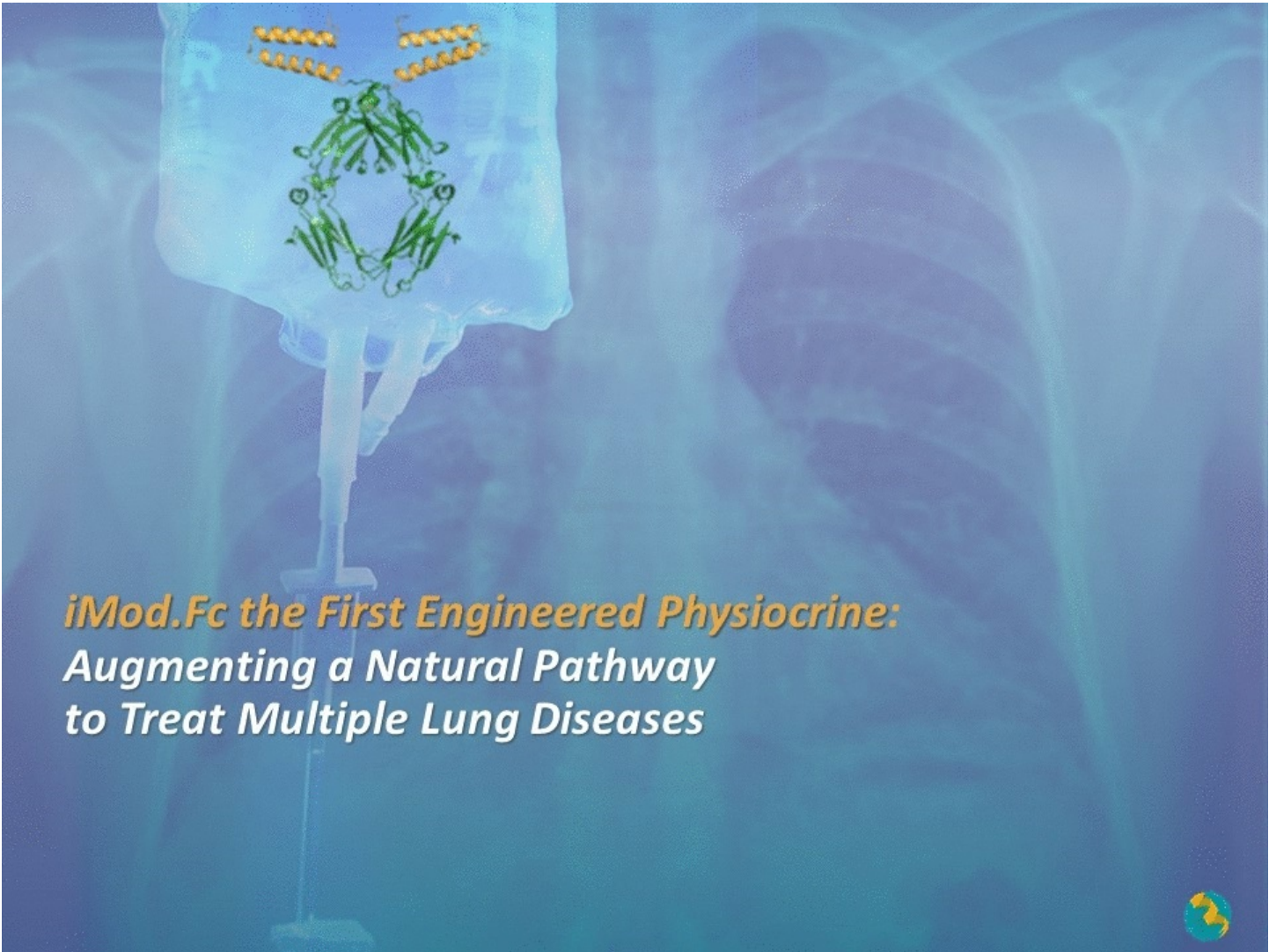
Promise for severely afflicted myopathy patients

COMMERCIAL
RATIONALE



Leadership position in FSHD clinical trials
Leverage registries, sites and advocacy
Common physician base

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 Kissel, Neural. Clin. 2014. 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



***iMod.Fc the First Engineered Physiocrine:
Augmenting a Natural Pathway
to Treat Multiple Lung Diseases***



An Engineered Physiocrine for Lung Disease: iMod.Fc

New TPP to open more lung indications

RESOKINE
FRANCHISE

Rationale for iMod.Fc*:

- Resolaris TPP: Weekly dosing; limits lung applications
- Develop molecule for new TPP: potentially once-monthly dosing

Product Concept:

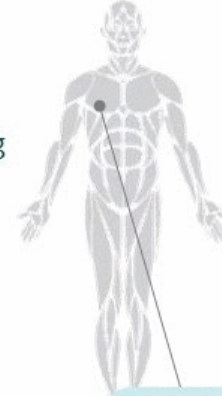
- Two iMod domains per Fc of an antibody
- Extend exposure to hit TPP

Preclinical Path and Goals:

- Industry proven model of IPF (approved drugs: Pirfenidone & Nintedanib)
- *E. coli* produced for low COGs
- Show immuno- & fibro- modulatory activity
- Show safety at higher doses

Potential Therapeutic applications:

- Rare pulmonopathies with an immune component (RPICs)
- Broader reach into RPICs and interstitial lung disease (ILD) indications



Rare Pulmonopathies with an Immune Component ("RPICs")



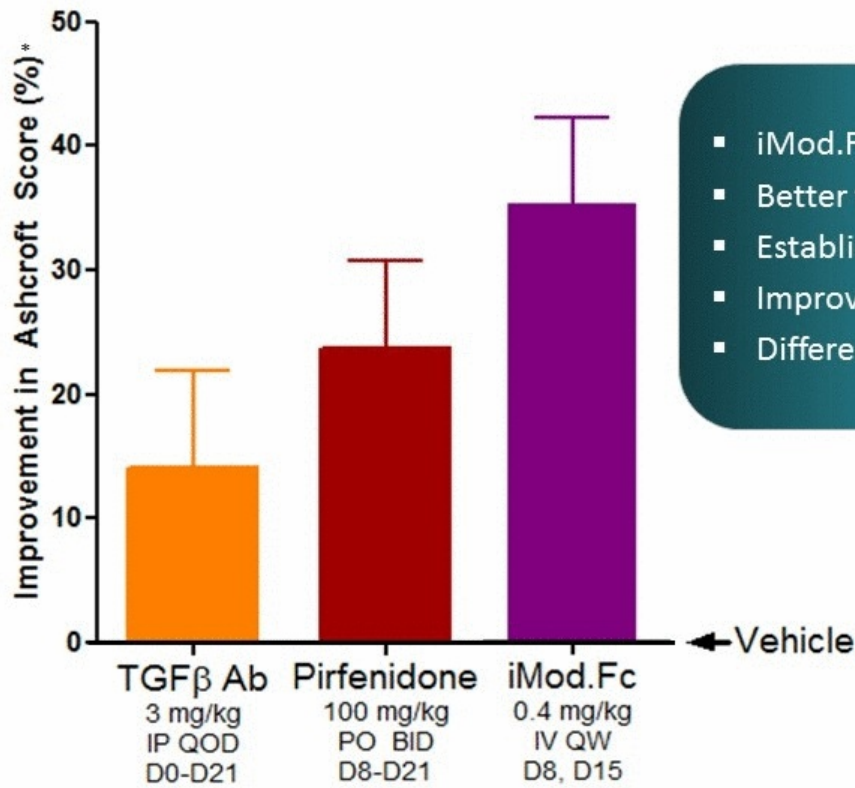
Lung

* iMod.Fc refers to immunomodulatory domain of HARS fused to an Fc region of an antibody
TPP = Target Product Profile



Two iMod.Fc Doses Outperform 28 Pirfenidone Doses

ATTRACTIVE
ILD CANDIDATE



*The Ashcroft scale for the evaluation of bleomycin-induced lung fibrosis is the analysis of stained histological samples by visual assessment

Non-Human Primates

Non-GLP double dose toxicology

- 1-month study at dose level 25x efficacious dose
- No pro-inflammatory cytokine signal
- No clinical observations
- No changes in body or tissue weights

Attractive PK

- >1nM for at least 500 hours at 1mg/kg

Rodents

Non-GLP toxicology

- 1-month study at dose level 25x efficacious dose
- No pro-inflammatory cytokine signal
- No clinical observations
- No changes in body or tissue weights

Attractive PK

Supports potential for monthly dosing in patients



iMod.Fc for Multiple Lung Indications

Severe, rare disorders with high unmet medical need

iMOD.Fc
CLINICAL

> 80 RPIC Forms
Including Interstitial Lung Diseases

Pathogenesis

- Lung damage leading to alveolar inflammation or fibrosis
- Worst prognosis: lower DLCO and rapid decline of DLCO over three years

Clinical manifestations

- Shortness of breath and cough
- Specific chest radiographic abnormalities
- Decreased lung volume noticed in pulmonary function tests

Standard of care

- O₂, pulmonary rehabilitation; lung transplant
- Immunosuppressive (cyclophosphamide with low dose prednisone)
- For IPF, Pirfenidone & Nintedanib

Upcoming Trials

- **Expect to initiate clinical trial with iMod.Fc in 2017**
- Evaluating appropriate forms of RPICs, including ILD
- Goal is to explore safety, tolerability, biological and clinical activity





***Building a New Class of Therapeutics:
Foundation for the Future***

Strong Cash Position and Focused Execution

Balance sheet well-aligned to achieve near-term catalysts

Milestones 2016/2017

Early Onset FSHD 003 Data
(4Q16)

Adult LGMD/Adult FSHD 004 Data
(4Q16)

Adult FSHD Long Term Extension 005 Data
(4Q16)

Initiate iMod.Fc Clinical Trials
(2017)

Financials

Cash as of 12/31/15:
\$125.3M

Shares Outstanding:
23.7M



Revolutionary Drugs Leveraging New Biology

Opportunity to own a new class of meaningful medicines

MEANINGFUL
MEDICINES





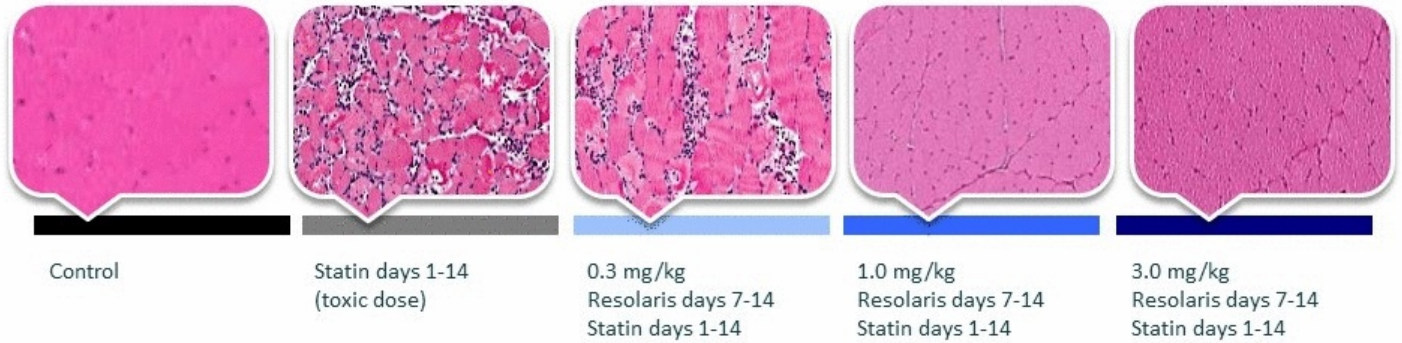
Appendix




Ability to Treat Immune Cell Invasion in Muscle

Myopathy model: *in vivo* Resolaris effects

RESOLARIS
PRECLINICAL

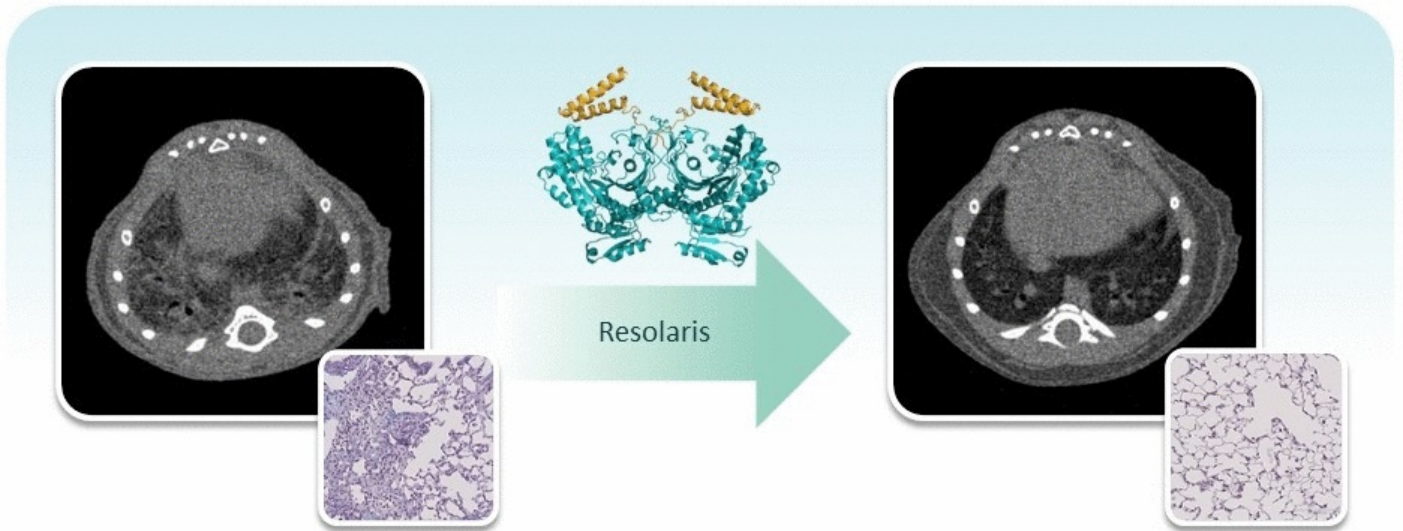



 ↓ Cytokines, ↓ T-cells and ↓ Monocytes with Resolaris administration

Resolaris: Active In Lung Inflammation & Fibrosis Model

Three week rodent model, two weeks of therapeutic treatment






PROMISING
THERAPEUTIC
ACTIVITY



 Pulmonary Inflammation and Fibrosis Induced with Bleomycin
Promising therapeutic activity*
Compared favorably to Pirfenidone

Experimental data provided by Stelic CRO
CT scans taken at day 14, lung histology taken at day 21
* Activity of mouse Resolaris (3mg/kg) vs vehicle control

FSHD Molecular Pathology Links Loss of Epigenetic Control to Immune Status with Disease

FSHD Muscle Phenotype	4th Chromosome Terminal Repeats DUX 4	Non-Germline Gene Expression	Skeletal Muscle Result	Immune Cell Invasion	Disease Status
 <p>Normal</p>	 <p>Full Epigenetic Control ~100 repeats</p>	Silent	Normal	No	Normal
<p>FSHD</p> 	 <p>Partial Epigenetic Control</p>	Activatable	<p>↑DUX 4</p> <p>↑“Non-muscle” proteins</p>	Yes	Moderate to Severe
<p>Most Severe Typically</p>	 <p>Greatest Loss of Epigenetic Control</p>	Highly Activatable	<p>↑DUX 4</p> <p>↑“Non-muscle” proteins</p>	Yes	Severe

Statland, J., C. M. Donlin-Smith, et al. *Journal of Neuromuscular Diseases*, 2014. Lemmers, E. et al, *Gene Reviews* 2014
 | = D4Z4 Repeat (containing DUX4)

