
**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

May 11, 2016
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))
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Item 2.02 Results of Operations and Financial Condition.

On May 11, 2016, aTyr Pharma, Inc. (the “Company”) announced financial results for the quarter ended March 31, 2016 in the earnings release attached hereto as Exhibit 99.1.

The information under this Item 2.02, including Exhibit 99.1 hereto 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 (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall such information be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended (the “Securities Act”) or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01 Regulation FD Disclosure.

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 99.2. The Company does not undertake to update the presentation materials.

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

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

- 99.1 Earnings Press Release of aTyr Pharma, Inc. dated May 11, 2016.
- 99.2 Corporate Presentation Materials of aTyr Pharma, Inc. dated May 2016.

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: May 11, 2016

INDEX TO EXHIBITS

- 99.1 Press release of aTyr Pharma, Inc. dated May 11, 2016.
- 99.2 Corporate Presentation Materials of aTyr Pharma, Inc. dated May 2016.

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

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aTyr Pharma Announces First Quarter 2016 Operating Results

Company Continues Development Strategy Leveraging Resokine Pathway

SAN DIEGO – May 11, 2016 – 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 operating results for the first quarter ended March 31, 2016.

First Quarter Results

Research and development expenses were \$12.0 million and \$6.6 million for the quarters ended March 31, 2016 and 2015, respectively. The increase of \$5.4 million was due primarily to a \$5.8 million increase related to manufacturing costs and clinical and non-clinical development costs incurred in support of various activities for Resolaris™ and a \$0.9 million increase related to compensation expenses resulting from increased headcount in research and development functions, including \$0.2 million in non-cash stock-based compensation. The increase was offset by a decrease related to a one-time \$1.4 million non-cash expense for the assignment of certain intellectual property rights in the prior year period.

General and administrative expenses were \$4.1 million and \$2.3 million for the quarters ended March 31, 2016 and 2015, respectively. The increase of \$1.8 million was due primarily to a \$1.6 million increase in personnel costs resulting from increased headcount inclusive of \$0.5 million in non-cash stock-based compensation.

Recent Highlights

The company is also pleased to announce that it has completed enrollment of its Phase 1b/2 clinical trial testing Resolaris in patients with limb-girdle muscular dystrophy 2B (LGMD2B or dysferlinopathies) or facioscapulohumeral muscular dystrophy (FSHD). Data from this clinical trial is expected to be announced in the fourth quarter of this year.

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 modulators. The Company's lead candidate, Resolaris™, is a potential first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component. Resolaris is currently in a Phase 1b/2 clinical trial in adult patients with facioscapulohumeral muscular dystrophy (FSHD); a Phase 1b/2 trial in adult patients with limb-girdle muscular dystrophy 2B (LGMD2B or dysferlinopathies) or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. To protect this pipeline, aTyr built an intellectual property estate comprising over 70 issued or allowed patents and over 240 pending patent applications that are owned or exclusively licensed by aTyr, including over 300 potential Physiocrine-based protein compositions. aTyr's key programs are currently focused on severe, rare diseases characterized by immune dysregulation for which there are currently limited or no treatment options. For more information, please visit <http://www.atyrpharma.com>.

Forward-Looking Statements

This press release 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 of Resolaris or iMod.Fc, the ability of the Company to undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals, and the timing of initiation of additional clinical trials 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, 2015 and in our subsequent 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.

ATYR PHARMA INC.
Condensed Consolidated Statements of Operations
(unaudited, in thousands, except share and per share data)

	Three Months Ended	
	March 31,	
	2016	2015
Operating expenses:		
Research and development	\$ 12,000	\$ 6,593
General and administrative	4,115	2,329
Total operating expenses	16,115	8,922
Loss from operations	(16,115)	(8,922)
Other income (expenses), net	28	(149)
Net loss	(16,087)	(9,071)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.68)	\$ (9.39)
Weighted average shares outstanding, basic and diluted	23,631,133	966,322

ATYR PHARMA INC.
Condensed Consolidated Balance Sheets
(in thousands)

	March 31,	December 31,
	2016	2015
	(unaudited)	
Cash, cash equivalents and investments	\$ 111,605	\$ 125,349
Other assets	1,675	2,533
Property and equipment, net	1,845	1,793
Total assets	\$ 115,125	\$ 129,675
Accounts payable, accrued expenses and other liabilities	\$ 10,370	\$ 9,483
Current portion of commercial bank debt	3,427	3,366
Commercial bank debt, net of current portion	896	1,776
Stockholders' equity	100,432	115,050
Total liabilities and stockholders' equity	\$ 115,125	\$ 129,675

###



**BUILDING A NEW CLASS OF MEDICINES
PHYSIOCRINE BASED THERAPEUTICS**

**1ST RARE DISEASE TRIAL COMPLETED
NEW HOPE FOR MUSCULAR DYSTROPHY PATIENTS**

May 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 and in our subsequent 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 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
- Therapeutically dose above normal levels to promote tissue homeostasis

Resolaris: Clinical Development Program

- 3 ongoing trials in muscular dystrophies
- 1st muscular dystrophy trial completed
- 1st potential activity signal in FSHD
- 1st safety & tolerability data in patients

Autonomous Pipeline & Unique Business Model

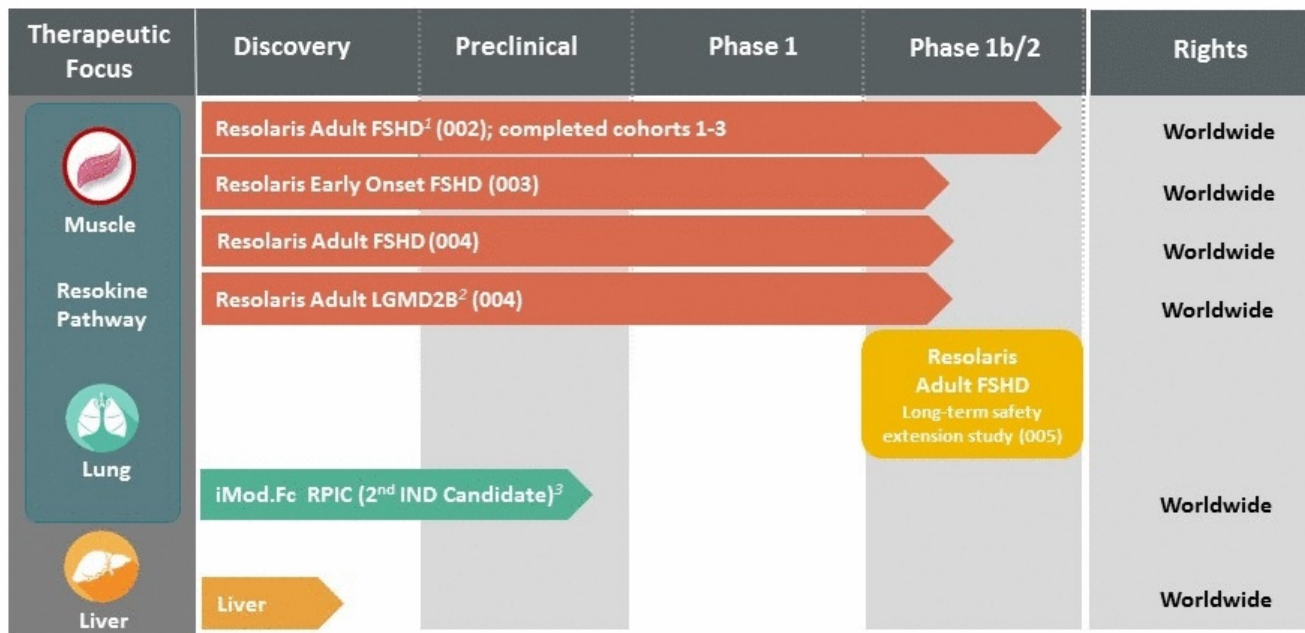
- 1st in class biologics pipeline
- 2nd molecule for lung disease
- Build franchises in muscle, lung & liver based on new biology

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

Harnessing the Power of Physiocrines for Patients

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

ATYR
PIPELINE



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 2B

³ RPIC: Rare pulmonopathies with an immune component, including Interstitial Lung Disease ("ILD")

Humira® WW sales over \$14B in 2015 (TNF inhibitor)

Insulins used by over 3 million Americans to treat diabetes in 2012

Complement inhibitors being studied in a dozen ongoing clinical trials

Soliris® net product sales over \$2.6B in 2015 (complement inhibitor)

POTENTIAL OF PHYSIOCRINE PROTEINS
DISCOVERY OF A NEW CLASS OF PROTEINS FROM
ALTERNATIVE SPLICING OF ANCIENT GENES

Science 1999 **Nature 2010** **Nature 2013** **Science 2014** **Nature 2015**

~300 proteins involved in physiological pathways, >70 issued or allowed 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

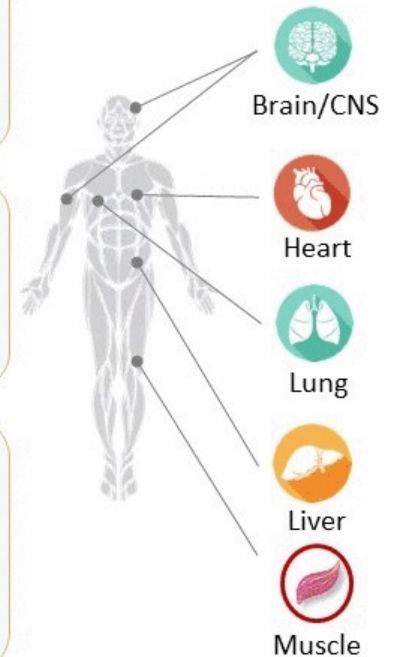
iMod.Fc focused on rare lung diseases with an immune & fibrotic component

- At least 14/23 tRNA Synthetases in family w/ known disease connections
- Clinical phenotypes present tissue-by-tissue homeostasis

- HARS* genetic syndrome (“HGS”) and anti-synthetase syndrome (Jo-1 Ab to HARS)
- Disease phenotype: effects skeletal muscles and lung
- Potentially deficient pathway

Genetic disease paradigm:

- Genetic aberration → protein absence or abnormality → abnormal function/structure → immune system engagement
- Soliris® (\$2.6B in 2015): example of therapeutic altering the immune component in diseases with genetic aberration



*histidyl-tRNA synthetase



HARNESSING THE RESOKINE* PATHWAY
NATURAL PATHWAY FROM SKELETAL MUSCLE
TO TREAT MULTIPLE RARE MUSCLE DISEASES

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

Model of Resokine Pathway

In skeletal muscle health and disease

RESOKINE
PATHWAY

IGF induced growth
Muscle progenitor cell

Resokine^A release



Resokine release



Free Resokine^A circulating levels ~100 pM



Anti-synthetase syndrome
free Resokine^A <10 pM (lung/muscle phenotype)
HARS genetic syndrome (lung/muscle phenotype)

Normal damage

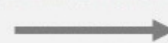


+



Immune cells

Immune cell invasion/residence



=



Normal resting muscle

Our Hypothesis:

Severe damage (myopathies)



Genetic mutation

1. Structural protein
2. Transcription factors
3. Mitochondrial proteins

Auto-Immune
1. Immune system dysregulation

Agent Induced
1. Statins



FSHD¹, LGMD², DMD³
>30 different muscular dystrophies

+

Therapeutic Resolaris



10-100x's normal levels

=



Potentially healthier muscle

¹ Facioscapulohumeral Muscular Dystrophy

² Limb-girdle Muscular Dystrophy

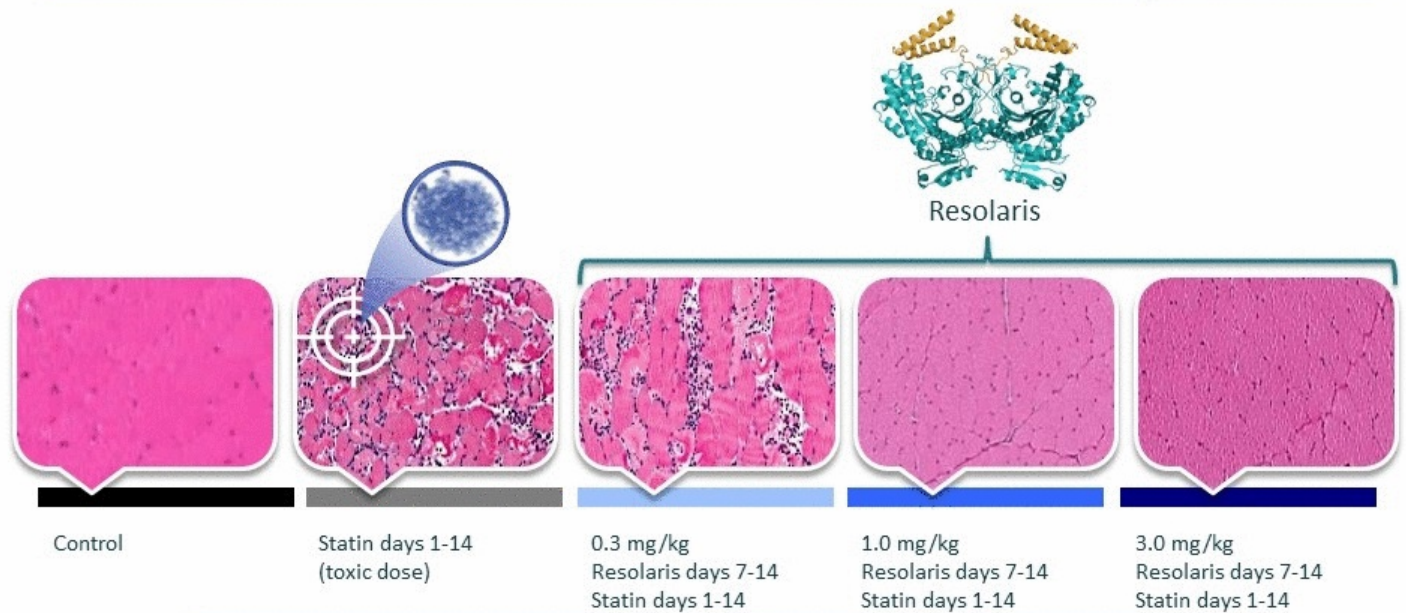
³ Duchenne Muscular Dystrophy

^A aTyr Pharma discovery

Treating Immune Cell Invasion in Skeletal Muscle

Two weeks of therapeutic treatment in Statin myopathy model

RESOKINE
PATHWAY



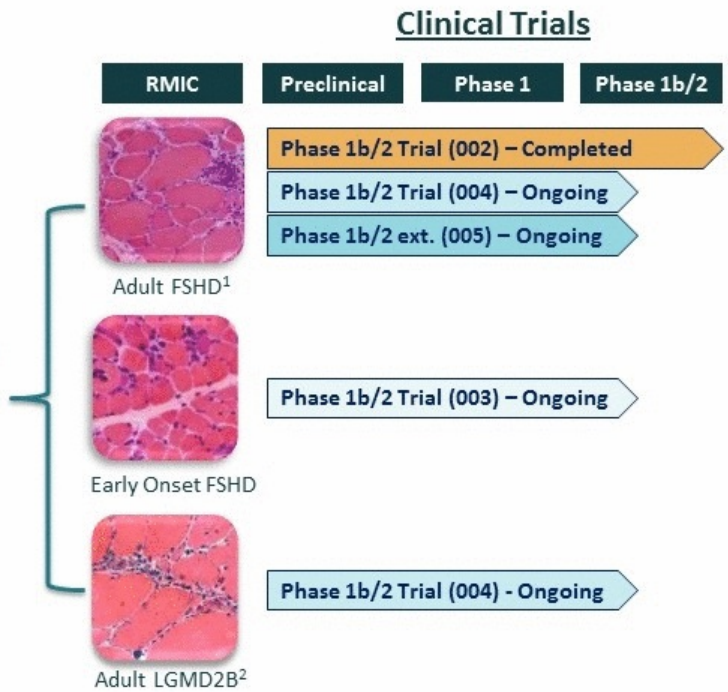
↓ Cytokines, ↓ T-cells and ↓ Monocytes
with Resolaris administration

Clinical Path for Resolaris in Skeletal Muscle

Staging rare muscle disease indications

RESOKINE
PATHWAY

- 1st Physiocrine based therapeutics to promote homeostasis
- Establish & explore:
 - Safety, tolerability
 - Activity/end-point potential
- Focus on Rare Myopathies with an Immune Component (RMIC)
- Multiple opportunities for advancement for patients with few or no treatment options



¹ Facioscapulohumeral Muscular Dystrophy

² Limb-girdle Muscular Dystrophy 2B



CLINICAL DEVELOPMENT OF RESOLARIS
POTENTIAL NEW THERAPIES FOR PATIENTS
WITH RARE MUSCLE DISEASES

FSHD: A Severe Skeletal Muscle Disease

Muscle-by-muscle disease progression

CLINICAL
DEVELOPMENT

Pathology

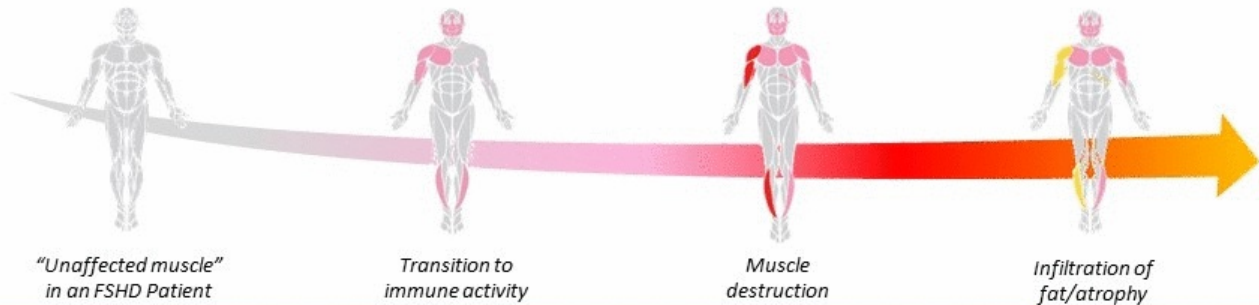
- Dominant/spontaneous toxic gain of function (\uparrow Dux4)
- Immune component (e.g. \uparrow T cells)
- Asymmetric muscle loss, fat infiltration

Clinical

- Debilitating muscle weakness
- Often diagnosed before adulthood
- May have visual or auditory impairment (early onset)

Standard of care

- No therapeutic treatments
- Only supportive care provided



Design

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

Objectives

Build dossier for Resolaris & new class

- Safety
- Tolerability
- Immunogenicity
- PK

Explore FSHD pertinent readouts

- Circulating markers of disease
- Targeted MRI of disease muscle
- Strength
- Patient reported outcomes

Across 3 dose cohorts over 1 or 3 months of dosing

Results

Completed 1st multiple dose trial

- ✓ Safety
- ✓ Tolerability*
- ✓ Immunogenicity
- ✓ PK

Completed 1st FSHD only trial

- ± Only 2/20 patients with elevated levels
- ± Targeted MRI may be too narrow
- ✓ Muscle testing may hold promise
- ✓ Potential signal to confirm

Potential active dose:

3.0 mg/kg (weekly) @ 3 months

**One reversible Infusion Related Reaction (IRR) patient in 002 & two reversible IRR patients in 005 trial*

Establishing the Safety Profile: 1st FSHD Trial

First Physiocrine therapeutic assessed in patients

CLINICAL
DEVELOPMENT

- ✓ 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

- ✓ ADA*'s were of low titer and no demonstrated effect on pharmacokinetics of Resolaris

- ✓ Procedures in effect to minimize IRRs/ADAs moving forward

001/002 trials collectively yield safety data from 39 subjects

*ADA: Anti-drug antibody

15

 aTyr Pharma

Global Patient Reported Outcomes: INQoL

Individualized neuromuscular quality of life assessment

CLINICAL
DEVELOPMENT

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

- Life Domains
 - I) Activities II) Independence III) Social Relationships IV) Emotions V) Body Image
- Symptoms, Treatment Effects
- 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.”***

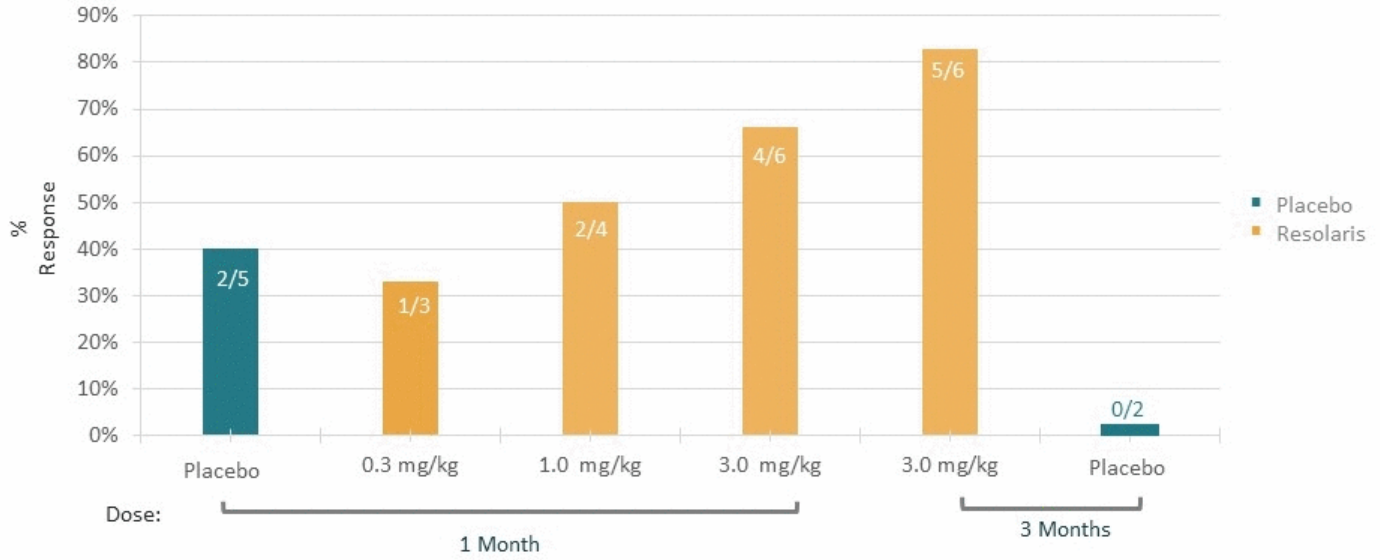
* Vincent KA et al: Construction and Validation of a Quality of Life Questionnaire for Neuromuscular Disease (INQoL). *Neurology* 2007, 68:1051-1057.

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

Proportion of Patients with Improved INQoL Overall Scores

Potential dose response and duration response

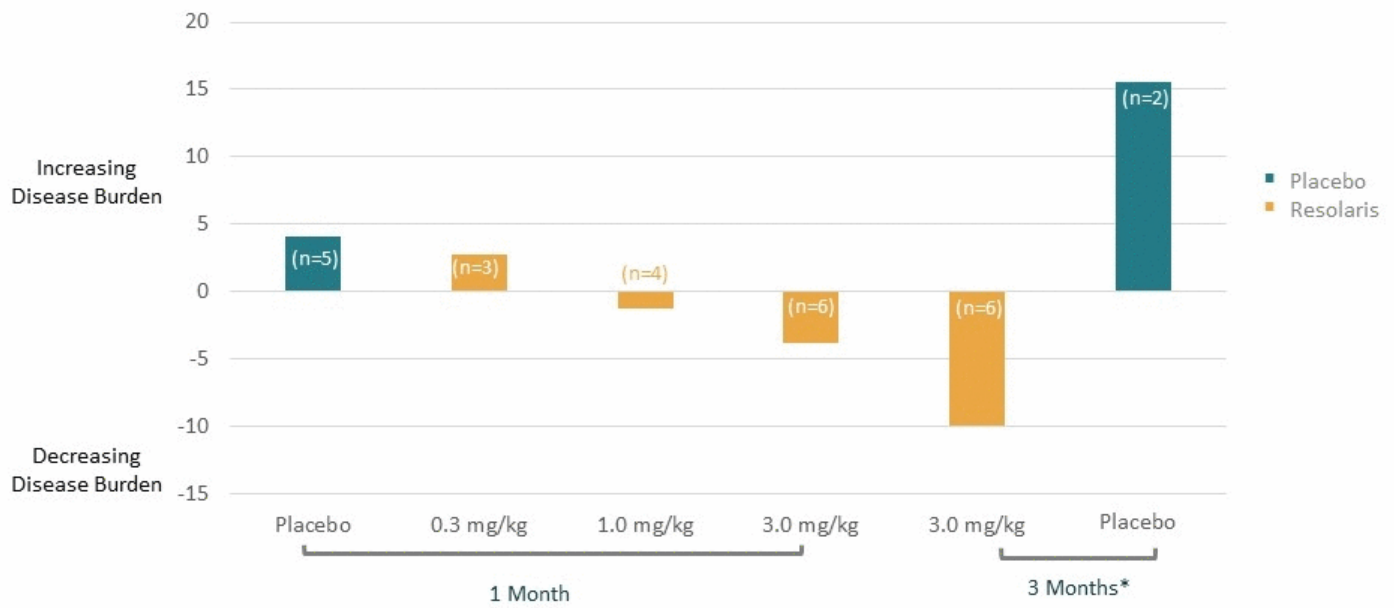
CLINICAL
DEVELOPMENT



Multiple Dimensions Improve on INQoL

INQoL Overall Scores Change from Baseline (%); ITT Population (n=20)

CLINICAL
DEVELOPMENT



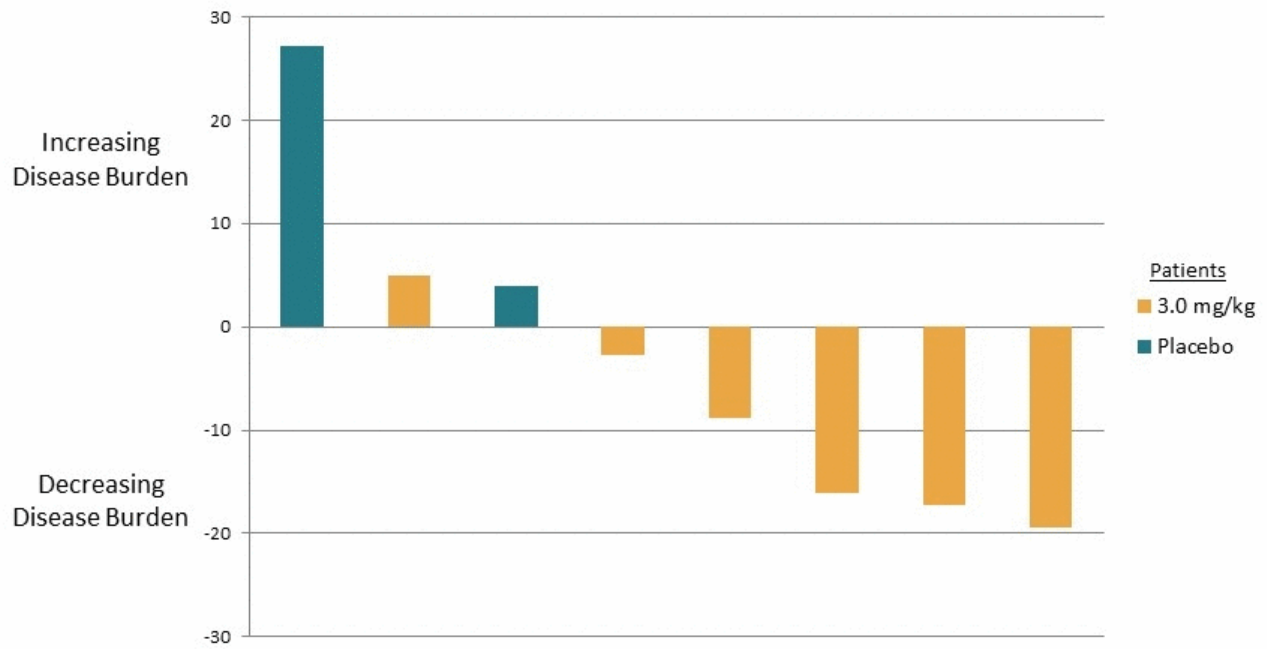
Trial not powered to show statistical significance.
Data suggest potential improvement in this relatively small clinical trial of FSHD patients.

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

Overall INQoL Score Placebo v. 3.0 mg/kg Patients

Suggestive of patient improvement in 3 months

CLINICAL
DEVELOPMENT



Testing muscle function/strength

- 15 muscles evaluated at 4 time points in study
- Muscles scored individually
- Composite score calculated
- Common endpoints for particular muscles



Data suggest small trends of slower progression or potential improvement

Limb-Girdle Muscular Dystrophy 2B (LGMD2B)

A severe muscle disease with a genetic loss of function

CLINICAL
DEVELOPMENT

Pathology

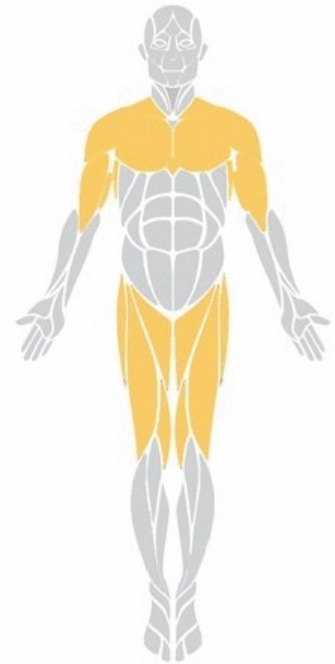
- Immune component (e.g. ↑ T-cells)
- Toxic loss of function mutation (dysferlin)
- Muscle group progression

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)

Already completed enrollment

CLINICAL
DEVELOPMENT

Purpose

- Evaluate the safety/tolerability of total weekly exposure of 6.0mg/kg in LGMD2B and FSHD
- Assess drug activity more proximal to dosing

Rationale

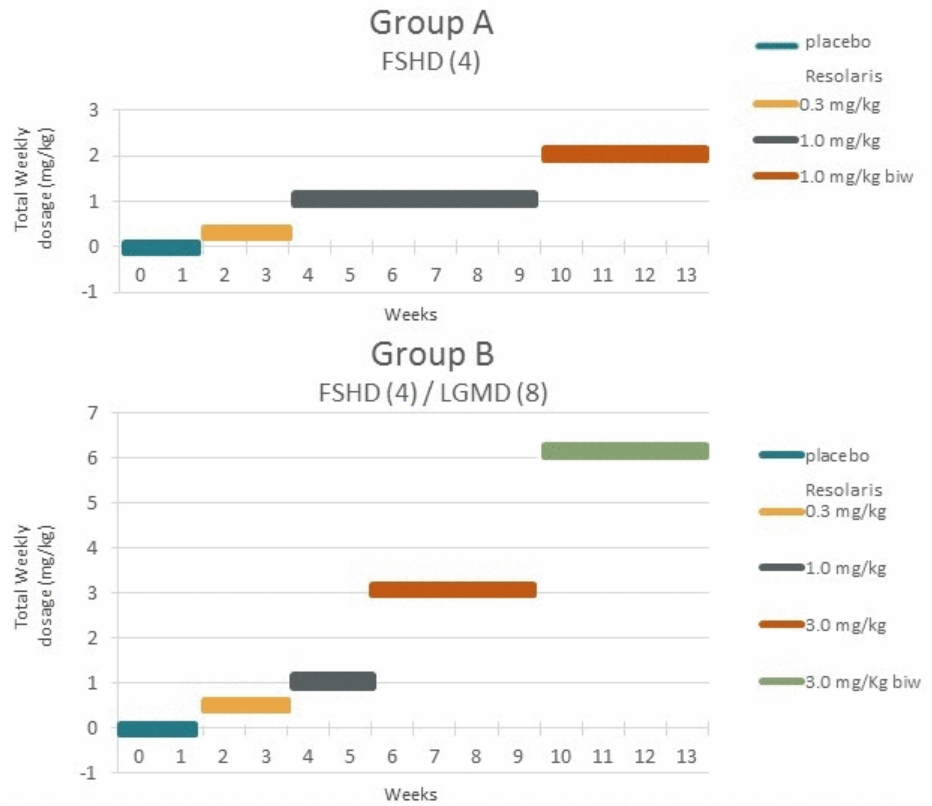
To test whether a total weekly exposure of 6.0mg/kg demonstrates different outcomes than a total weekly exposure of 3.0mg/kg

Design & Study Sites

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

Study Population / Entry Criteria

- 16 patients,
8 each with LGMD2B and FSHD,
18-75 years of age
- MRI positive or
 - Circulatory markers (in LGMD2B patients only)



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
				<ul style="list-style-type: none"> • 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

Purpose

- Evaluate the safety/tolerability in a potentially different indication Early Onset FSHD
- Assess drug activity in new patient population and with additional endpoints

Rationale

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

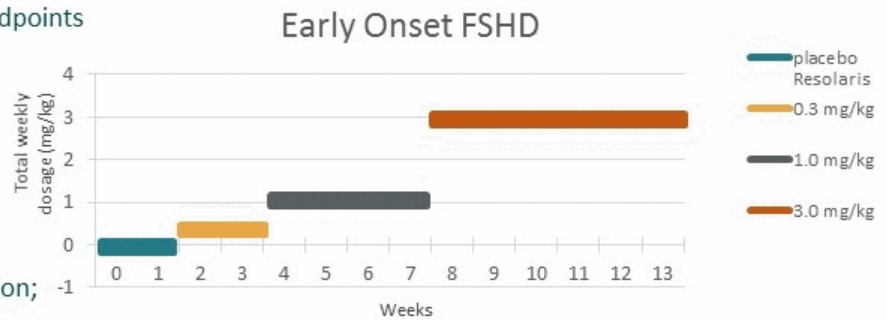
Design & Study Sites

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

Study Population / Entry Criteria

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

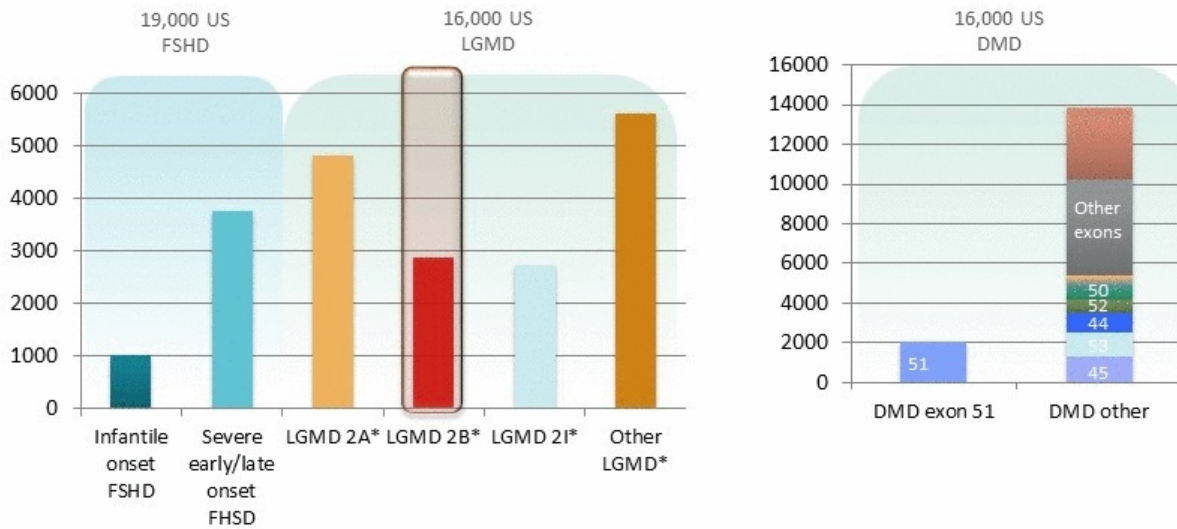
Enrollment ongoing in Stage 1



Resolaris: One Product, Multiple Rare Diseases

Promise for severely afflicted myopathy patients

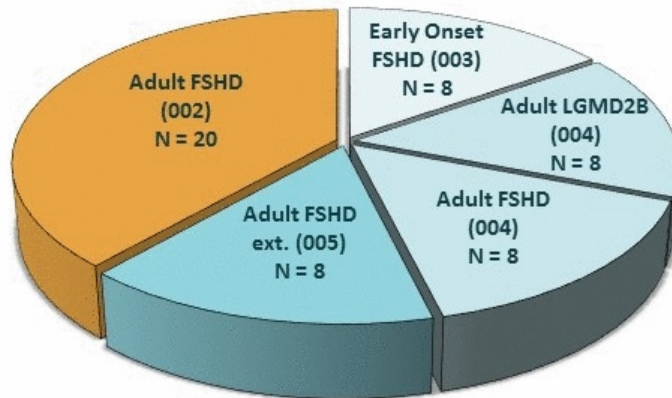
CLINICAL
DEVELOPMENT



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 Kisse, *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*

**Trials ongoing in three different RMICs,
enrolling ~24 additional patients in 2016**

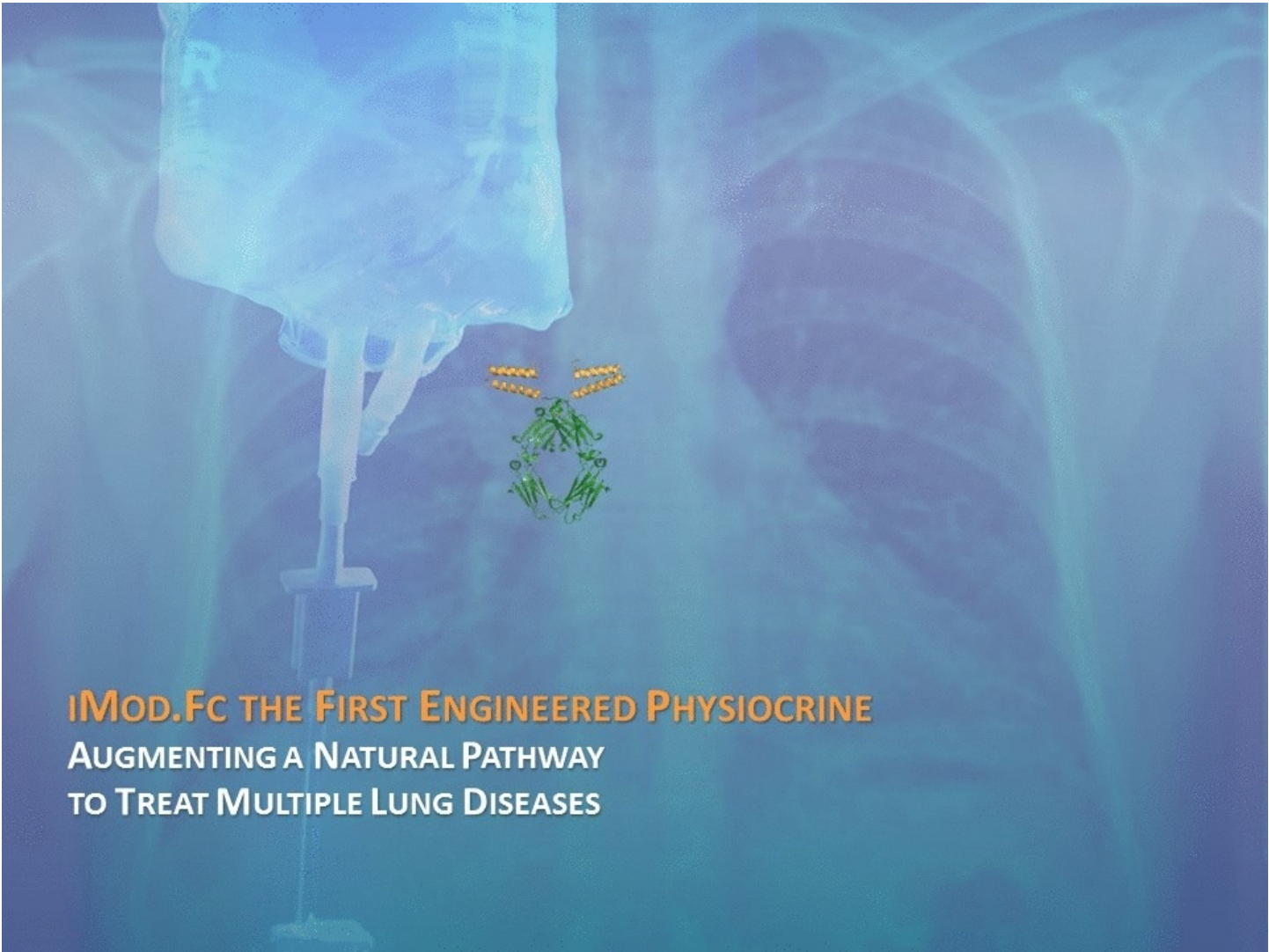


Status:

- ✓ 002 complete
- ✓ 004 enrollment complete
- 003 enrolling
- 005 ongoing long-term
- Additional data expected in 4Q from ongoing trials

Next trial(s) based on results of 002, 003, 004 & 005 trials

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



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 and new molecule to open up lung indications

IMOD.Fc
PROGRAM

Rationale for iMod.Fc*

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

Product Concept

- Two iMod domains per Fc of an antibody
- Extend exposure to hit TPP
- Modulating the immune and fibrotic pathways

Preclinical Status and Goals

- ✓ Successful *E. coli* production for low COGs
- ✓ Activity in industry proven model of IPF (approved drugs: Pirfenidone & Nintedanib)
- ✓ Immuno- & fibro- modulatory activity
- ✓ Rat/non-human primate safety and PK data supportive advancement to IND
- Expect to initiate clinical trial with iMod.Fc in 2017

Potential Therapeutic applications

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



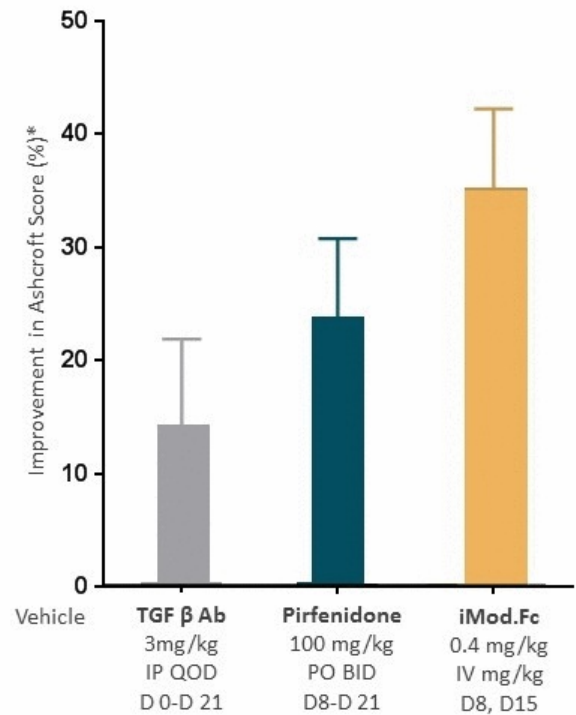
*iMod.Fc refers to immunomodulatory domain of HARS fused to an Fc region of an antibody
TPP = Target Product Profile

Two iMod.Fc Doses Outperform 28 Pirfenidone Doses

IPF Model Activity

iMOD.FC
PROGRAM

- iMod.Fc 1/250th of Pirfenidone dose
- Better than 10 TGF β Ab doses
- Established IPF rodent model
- Improves inflammation & fibrosis
- Differentiated mechanism



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



BUILDING A NEW CLASS OF THERAPEUTICS
FOUNDATION FOR THE FUTURE

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



Grove Matsuoka
SVP, Product Programs and Planning

CoDa Therapeutics, Inc.



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



John Blake, CPA
VP, Finance



Kelly Blackburn
VP, Clinical Operations



Ashraf Amanullah, Ph.D.
VP, Manufacturing

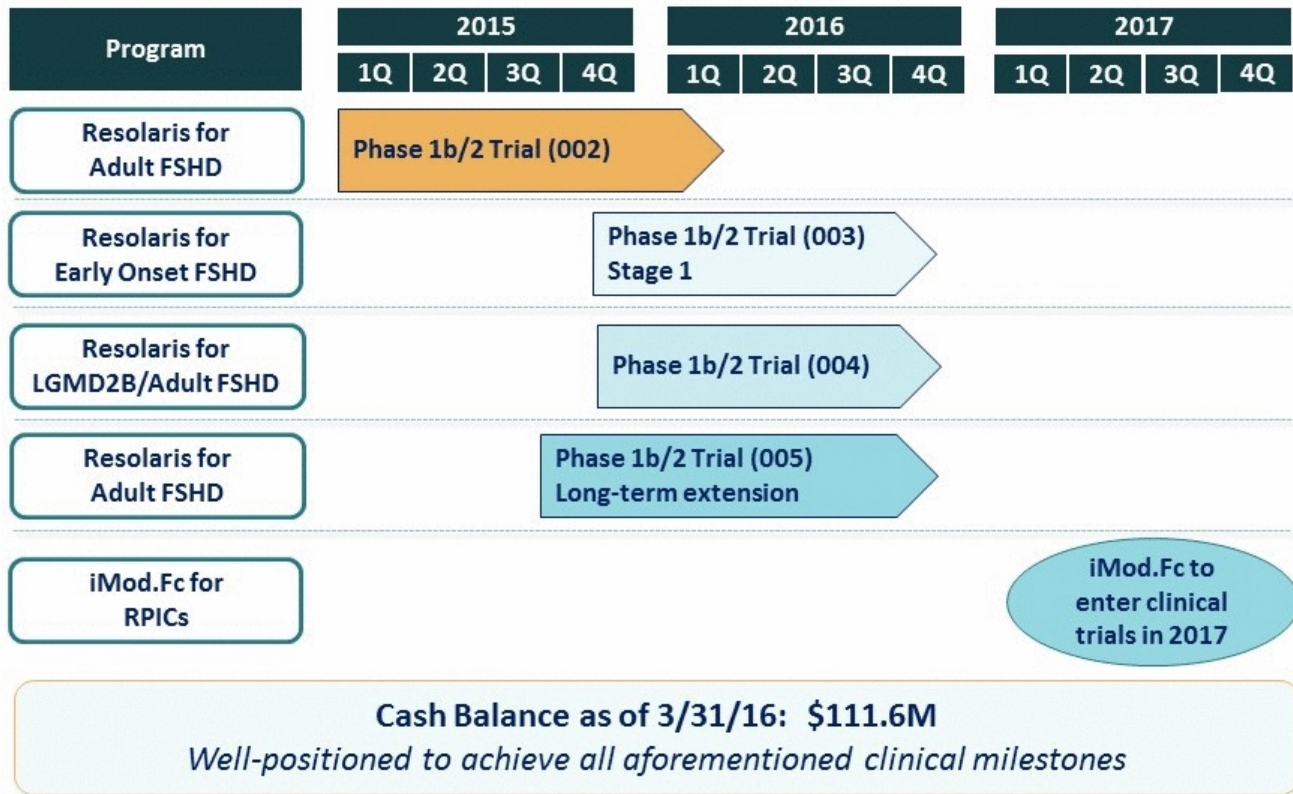


Holly D. Chrzanowski
VP, Enterprise Talent and Organization



Focused Execution with Strong Cash Position

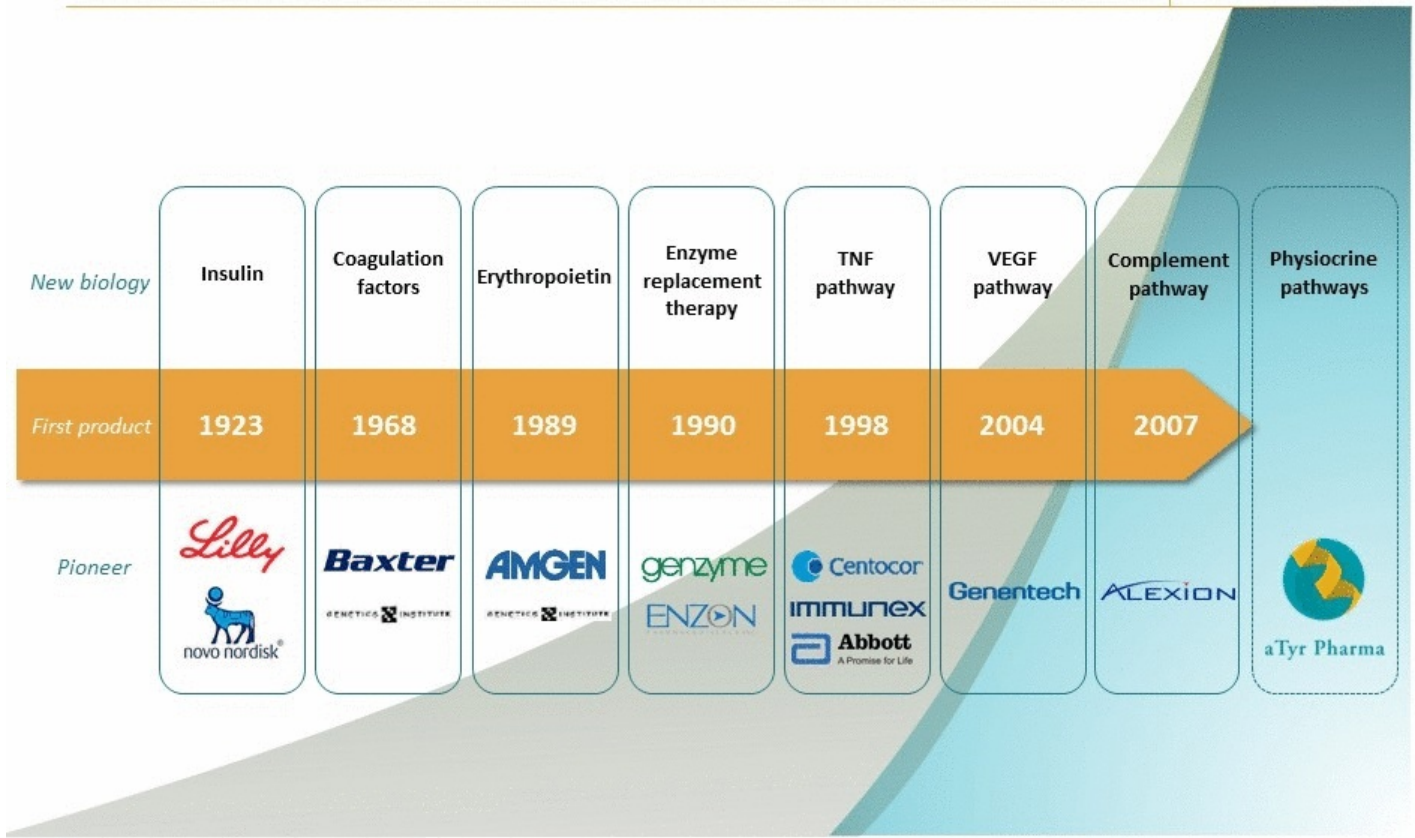
Balance sheet well-aligned to achieve near-term catalysts



Revolutionary Drugs Leveraging New Biology

Opportunity to own a new class of meaningful medicines

HISTORY AND FUTURE
OF BIOTECH





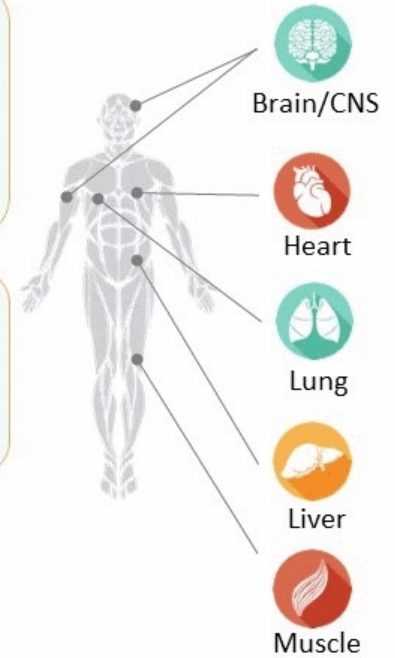
APPENDIX

Properties of Physiocrines






PHYSIOCRINE
PROTEINS

- 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



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>Most Severe Typically</p>	 <p>Partial Epigenetic Control</p>	Activatable	<p>↑DUX 4</p> <p>↑“Non-muscle” proteins</p>	Yes	Moderate to Severe
	 <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)

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.

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Targeted MRI & Circulating Markers

To be monitored in additional studies and extension phase

CLINICAL
DEVELOPMENT

- 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

Resolaris Phase 1b/2 Program Summary

CLINICAL
DEVELOPMENT

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 trial(s) based on results of 002, 003, 004 & 005 trials

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

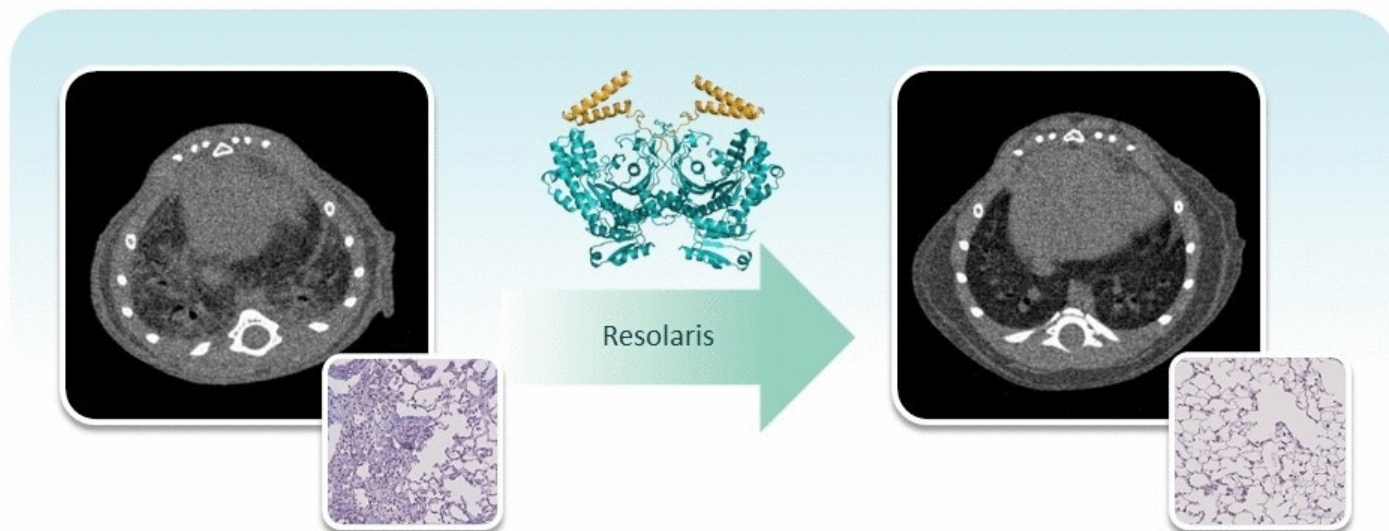
* Targeted MRI only if qualified with Stir+ muscle at baseline.


** Only for Cohort 3 subjects

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

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

> 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

