



# aTyr Pharma

## **aTyr Pharma Receives U.S. FDA Orphan Drug Designation for the Treatment of Limb Girdle Muscular Dystrophy with Resolaris™**

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SAN DIEGO, Feb. 28, 2017 /PRNewswire/ -- 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 that its product candidate Resolaris™ was granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of limb girdle muscular dystrophy (LGMD) patients.



"Receiving Orphan Drug Designation for the treatment of all types of LGMD patients with Resolaris is an important step in our overall process of bringing our first Physiocrine-based product candidate to the market to treat patients," said John Mendlein, CEO of aTyr Pharma. "This designation follows our recently granted Fast Track designation and our promising signals of clinical activity in a LGMD sub-type (LGMD2B). We are excited to continue the development of Resolaris to treat patients with rare myopathies with an immune component who currently have limited or no therapeutic treatment options."

The FDA's Orphan Drug Designation program is intended to advance the development of products which demonstrate promise in diagnosing or treating rare conditions that affect fewer than 200,000 people in the U.S. Sponsors developing orphan-designated products are eligible for incentives under the program, including seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and certain tax credits.

### **About Resolaris™**

aTyr Pharma is developing Resolaris as a potential first-in-class intravenous protein therapeutic 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 LGMD2B**

Limb girdle muscular dystrophy (LGMD) refers to a group of rare genetic myopathies, of which there are more than 20 different subtypes, none with approved therapies. LGMD affects an estimated 16,000 patients in the U.S., approximately 3,000 of whom have LGMD2B. LGMD2B is a recessive genetic disease caused by a toxic loss of function in the dysferlin gene. Patients experience progressive debilitating muscle weakness and atrophy as well as immune cell invasion in the skeletal muscle. To learn more about LGMD2B please visit [www.jain-foundation.org](http://www.jain-foundation.org).

### **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. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 80 issued or allowed patents and over 230 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, 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 including our most recent Quarterly Report for the quarter ended September 30, 2016. 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.

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