# FDA Grants Fast Track Designation for aTyr's Resolaris™ to Treat Limb Girdle Muscular Dystrophy 2B and Removes Partial Clinical Hold for Resolaris

January 18, 2017
- First Reported Fast Track Designation for LGMD2B Treatment -

SAN DIEGO, Jan. 18, 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 Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of limb girdle muscular dystrophy 2B (LGMD2B), making it the first known therapeutic candidate for the treatment of LGMD2B to receive the designation. In addition, the FDA removed its partial clinical hold on a dosing ceiling for Resolaris in clinical trials.

"This Fast Track designation, which is granted to drug candidates addressing serious conditions and that demonstrate the potential to address unmet medical needs, represents another step forward for our first product candidate based on the Physiocrine pathway," said John Mendlein, PhD, CEO of aTyr Pharma. "Combined with our Phase1b/2 data in LGMD2B, adult facioscapulohumeral muscular dystrophy (FSHD) and early onset FSHD patients, we believe we are building a clinical and regulatory foundation for future development of Resolaris to treat patients across multiple rare genetic myopathies with an immune component."

aTyr previously announced results from a completed Phase 1b/2 open-label, intra-patient dose escalation trial testing doses of Resolaris up to 3.0 mg/kg biweekly in patients with LGMD2B. Based on the clinical trials completed to date, Resolaris has demonstrated a favorable safety profile without signs of immuno-suppression of circulating immune cells. 78% of the LGMD2B patients in the trial (7 of 9) recorded increases in their muscle function at 14 weeks as measured by manual muscle test (MMT), a validated assessment tool. Overall, the LGMD2B patients had a mean increase of MMT scores from baseline of 6.2%.

"We appreciate the FDA's responsiveness to our request to remove the partial clinical hold that provides dosing flexibility based on our data for Resolaris," commented Sanjay Shukla, MD, MS, Chief Medical Officer of aTyr Pharma. "We also believe that during our safety and dose ranging Phase 1b/2 clinical trials we have potentially identified a dose for the next phase of clinical development with a favorable safety profile and potential clinical activity across different rare muscle indications."

**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.

**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.


| **Contact:** |
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| **Mark Johnson** |
| Sr. Director, Investor Relations |
| mjohnson@atyrpharma.com |
| 858-223-1163 |


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