



aTyr Pharma Receives EMA Orphan Drug Designation for the Treatment of Limb Girdle Muscular Dystrophy with Resolaris™

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SAN DIEGO, March 3, 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 the European Medicines Agency (EMA) has granted orphan drug designation to Resolaris™ for the treatment of limb girdle muscular dystrophy (LGMD) patients.



"As the week of International Rare Disease day concludes, we remind ourselves of our important mission to our patients – to develop new medicines based on our pioneering efforts in Physiocrine biology that we hope form the basis of meaningful changes to the lives of patients impacted by rare diseases worldwide. We are encouraged that the EMA has recognized the potential of Resolaris for LGMD patients," said John Mendlein, CEO of aTyr Pharma. "The Resolaris program has now been granted Orphan Drug Designation for both LGMD and facioscapulohumeral muscular dystrophy (FSHD) from the FDA and the EMA, as well as Fast Track designation for both rare myopathies with an immune component from the FDA. We are pleased with the development of Resolaris from a clinical and regulatory standpoint. We look forward to continuing to develop this potential therapy to help treat patients with these rare myopathies, for which there are limited or no treatment options, across the globe."

EMA orphan designation is intended to encourage development of medicines for the diagnosis, treatment, or prevention of life-threatening or chronically debilitating conditions that affect no more than 5 in 10,000 people in the EU. Medicines that receive orphan designation are eligible for a number of incentives, including assistance with development of the medicine; reduced fees for marketing-authorization applications; and extended market exclusivity once the medicine is authorized. Orphan designation is conferred following a positive opinion by the EMA's Committee for Orphan Medicinal Products (COMP).

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 pathways. 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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