Avidity Biosciences Honors Rare Disease Day®

Company plans to have three distinct rare disease programs in clinical development by the end of 2022

SAN DIEGO, Feb. 28, 2022 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™), today announced its support for Rare Disease Day® and highlights the importance of raising awareness about rare diseases and their impact on people's lives.

"We are committed to improving the lives of people living with rare diseases and strive to incorporate patient and family voices into everything we do," said Sarah Boyce, president and chief executive officer. "Today, we are hosting a panel of people living with or caring for someone with rare muscle diseases to hear about their journey to diagnosis and the impact of living with a rare disease. We look forward to our continued partnership with the patient and advocacy communities as we advance our development programs with the aim of having three clinical programs for three different rare diseases by the end of this year."

Avidity is currently running the Phase 1/2 MARINA™ trial of AOC 1001 for adults with myotonic dystrophy type 1 (DM1). The company is on track for AOC 1020 to treat facioscapulohumeral muscular dystrophy (FSHD) and AOC 1044 to treat Duchenne muscular dystrophy (DMD) to enter clinical studies in 2022.

"More than 30 million Americans have one or more rare diseases. Less than 10% of the 7,000-10,000 known rare diseases have an FDA approved therapy today. We need patient advocates now more than ever to continue to make their voices heard by policy makers. They have been, and always will be, the key to innovation," said Julia Jenkins, EveryLife Foundation Executive Director. "We are grateful for companies like Avidity who are committed to listening and learning from the patient community and for their continued dedication to the research and development of new treatments for people living with rare diseases."

Rare Disease Day takes place on the last day of February each year with the goal to raise awareness of many different rare diseases worldwide. EURORDIS established Rare Disease Day and coordinates with 65+ national alliance patient organization partners each year to honor those living with rare diseases.

About Myotonic Dystrophy Type 1
Myotonic dystrophy type 1 (DM1) is an underrecognized, progressive and often fatal disease caused by a triplet-repeat in the DMPK gene, resulting in a toxic gain of function mRNA. The disease is highly variable with respect to severity, presentation and age of onset, however all forms of DM1 are associated with high levels of disease burden and may cause premature mortality. DM1 primarily affects skeletal and cardiac muscle, however patients can suffer from a constellation of manifestations including myotonia and muscle weakness, respiratory problems, fatigue, hypersomnia, cardiac abnormalities, severe gastrointestinal complications, and cognitive and behavioral impairment. Currently, there are no treatments for people living with DM1.

About Facioscapulohumeral muscular dystrophy (FSHD)
Facioscapulohumeral muscular dystrophy (FSHD) is characterized by progressive and often asymmetric skeletal muscle loss that initially causes weakness in muscles in the face, shoulders, arms and trunk and progresses to weakness in muscles in lower body. FSHD is an autosomal dominant genetic disease, meaning a single copy of the disease-associated gene, DUX4 (double homeobox 4), is enough to cause the disease. The abnormal expression of DUX4 leads to a series of downstream events that result in skeletal muscle wasting and compromised muscle function, including an inability to lift arms for more than a few seconds, loss of ability to show facial expressions and serious speech impediments. These symptoms cause many people affected by FSHD to become dependent on the use of a wheelchair for mobility. Currently there are no approved treatments for people living with FSHD.

About Duchenne muscular dystrophy (DMD)
Duchenne muscular dystrophy (DMD) causes a lack of functional dystrophin that leads to stress and tears of muscle cell membranes, resulting in muscle cell death and the progressive loss of muscle function. The dystrophin protein maintains the integrity of muscle fibers and acts as a shock absorber through its role as the foundation of a group of proteins that connects the inner and outer elements of muscle cells. People living with DMD suffer from progressive muscle weakness that typically starts in boys at a very young age. Those living with the condition often require special aid and assistance throughout their lives and have significantly shortened life expectancy. While there are treatments approved to treat people with DMD, it remains a very high unmet need.

About Avidity Biosciences
Avidity Biosciences, Inc.'s mission is to profoundly improve people's lives by delivering a new class of RNA therapeutics - Antibody Oligonucleotide Conjugates (AOCs™). Avidity's proprietary AOCs are designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to target the root cause of diseases previously untreatable with RNA therapeutics. Avidity is on track to have three programs in clinical development by the end of 2022. The company's lead product candidate, AOC 1001, is designed to treat patients with myotonic dystrophy type 1 (DM1). AOC 1001 has

commenced clinical testing with the ongoing Phase 1/2 MARINA™ trial in adults with DM1. The next programs in the company's advancing and expanding pipeline are AOC 1044, the lead of three programs for the treatment of DMD, and AOC 1020, designed to treat people living with FSHD. Avidity anticipates both programs will enter the clinic by the end of 2022. Avidity is also broadening the reach of AOCs beyond muscle tissues through both internal discovery efforts and key partnerships as the company continues to deliver on the RNA revolution. Avidity is headquartered in San Diego. For more information about our science, pipeline and people, please visit www.aviditybiosciences.com and engage with us on LinkedIn and Twitter.

Forward-Looking Statements
Avidity cautions readers that statements contained in this press release regarding matters that are not historical facts are forward-looking statements and cautions readers not to place undue reliance on these forward-looking statements. These statements are based on the company's current beliefs and expectations, and the company undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date hereof. Such forward-looking statements include, but are not limited to, statements regarding: the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and timing of ongoing and planned clinical trials; and the broad potential of AOCs to treat rare and serious diseases. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of these plans will be achieved. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in the business, including, without limitation: Avidity is early in its development efforts; Avidity's approach to the development of product candidates based on its AOC platform is unproven, and the company does not know whether it will be able to develop any products of commercial value; potential delays in the commencement, enrollment and completion of clinical trials; the success of its preclinical studies and clinical trials for the company's product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; unexpected adverse side effects or inadequate efficacy of its product candidates that may limit their development, regulatory approval and/or commercialization, or may result in recalls or product liability claims; and other risks described in prior press releases and in filings with the Securities and Exchange Commission (SEC). All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995**.**

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