Avidity Biosciences Announces FDA Partial Clinical Hold on New Participant Enrollment in Phase 1/2 MARINA™ Trial

Participants currently enrolled in MARINA and MARINA-OLE™ trials may continue to be treated with AOC 1001

Avidity received Investigational New Drug (IND) clearance for FSHD and DMD studies from FDA; programs now advancing into the clinic

Company to host investor webcast today at 8:30 a.m. ET / 5:30 a.m. PT

SAN DIEGO, Sept. 27, 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 that the U.S. Food and Drug Administration (FDA) has placed a partial clinical hold on new participant enrollment in the Phase 1/2 MARINA™ clinical trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1). Close to 40 participants are currently enrolled in the MARINA and MARINA open label extension (MARINA-OLE™) trials.

All participants, whether they are on AOC 1001 or placebo, may continue in their current dosing cohort although no additional participants may be enrolled until the partial clinical hold is resolved. All participants in MARINA may roll over into the MARINA-OLE where they will receive AOC 1001 as planned. DM1 is an underrecognized, progressive and often fatal neuromuscular disease with no approved treatment options.

The partial clinical hold is in response to a serious adverse event reported in a single participant in the 4mg/kg cohort of the MARINA study. Avidity is working closely with the FDA and the trial investigator to assess the cause of this event. The company is taking all necessary steps to resolve the partial clinical hold on new participant enrollment as quickly as possible.

"The safety of participants enrolled in our clinical studies is our first priority. We are doing a thorough analysis and will work diligently with the FDA and the trial investigator to follow the progress of this participant and to resume new participant enrollment as soon as we can," said Sarah Boyce, president and chief executive officer at Avidity. "We share the sense of urgency with the DM1 community for effective therapies and we remain confident that AOC 1001 has the potential to address important unmet needs of people living with DM1. We want to thank each participant in the study, their families and the investigators for their continued contributions."

"We continue to look forward to the preliminary assessment of the MARINA study in Q4 and, with our two recent IND clearances, we are now advancing AOC 1020 for FSHD and AOC 1044 for DMD into the clinic this year as planned," added Ms. Boyce.

Avidity remains on track to conduct a preliminary assessment of safety, tolerability and key biomarkers in approximately half of the study participants in the MARINA trial in the fourth quarter of 2022.

Avidity received IND clearance from the FDA to proceed with the clinical trial of AOC 1020 for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and AOC 1044 for the treatment of Duchenne muscular dystrophy (DMD) with mutations amenable to exon 44 skipping. These programs are now advancing into the clinic.

Today's Webcast Information
Avidity's management team will host a webcast and conference call at 8:30 a.m. ET / 5:30 a.m. PT today, September 27, 2022. The live call can be accessed by dialing 866-652-5200 (US) and 412-317-6060 (International) and requesting Avidity Biosciences. A live webcast will also be available on the "Events and Presentations" page in the "Investors" section of Avidity's website. A replay of the webcast will be archived on Avidity's website following the event.

About Avidity
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 now has three programs in clinical development. The company's lead product candidate, AOC 1001, is designed to treat people with myotonic dystrophy type 1 (DM1). AOC 1001 is currently in Phase 1/2 development with the ongoing MARINA™ trial and MARINA-OLE™ 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. The FDA has cleared Avidity to proceed into the clinic with both AOC 1020 and AOC 1044. 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, CA. 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. These statements are based on the company's current beliefs and expectations. Such forward-looking statements include, but are not limited to, statements regarding: expectations for

Avidity's interactions with the FDA, the ongoing investigation into the underlying cause of the serious adverse event for the affected patient, and the anticipated impact of, and Avidity's ability to resolve, the partial clinical hold and resume enrollment in and complete the MARINA study, and to conduct and present data from the preliminary assessment of the MARINA study and the timing thereof; the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and timing thereof; and the potential of AOC 1001 to treat DM1. 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 may not be able to resolve the partial clinical hold and the analysis related to the underlying cause of the serious adverse event may result in delays in the MARINA study or an inability to compete the study; unexpected adverse side effects or inadequate efficacy of its product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in clinical holds, recalls or product liability claims; Avidity is early in its development efforts; Avidity's approach to the discovery and 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 preclinical studies or 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; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; regulatory developments in the United States and foreign countries, including acceptance of INDs and similar foreign regulatory filings and the proposed design of future clinical trials; disruption to its operations from the COVID-19 pandemic or the war in Ukraine; and other risks described in prior press releases and in filings with the Securities and Exchange Commission (SEC). Avidity cautions readers not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and the company undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date hereof. 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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