

## Avidity Biosciences to Present Oral Presentation on AOC 1001 for DM1 at American Academy of Neurology (AAN) 2021 Virtual Annual Meeting

LA JOLLA, Calif., April 16, 2021 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company pioneering a new class of oligonucleotide-based therapies called Antibody Oligonucleotide Conjugates (AOCs™), today announced that preclinical data for their lead candidate, AOC 1001 in development for myotonic dystrophy type 1 (DM1), will be presented at the American Academy of Neurology (AAN) 2021 Virtual Annual Meeting being held April 17-22, 2021.

The abstract on AOC 1001 was chosen by the AAN Science Committee as one of 11 abstracts to be highlighted in the Emerging Science Session.

"We are honored to be part of the Emerging Science session at such a prestigious meeting and are grateful to the AAN Science Committee for highlighting AOC 1001. AOC 1001 is targeting DM1, a complex disease with variability from patient to patient and no approved treatments," said Art Levin, Ph.D., Chief Scientific Officer. "Our data presented at AAN show that AOC 1001 is highly potent with demonstrated activity in the nucleus and cytoplasm and that single doses of AOC 1001 produced 75% reductions in DMPK mRNA expression that were maintained for months post-dosing. These results further solidify AOC 1001 as a promising program with the potential to be the first therapy for people living with DM1."

Details of the oral presentation are below:

**Title:** "Optimization of AOC-1001, an antibody-oligonucleotide conjugate targeting the underlying cause of myotonic dystrophy type 1"

**Date:** Sunday, April 18, 2021 at 3:45 p.m. ET

**Session:** Emerging Science

**Presenter:** Barbora Malecova, Ph.D., Associate Director, Biology at Avidity

Avidity scientists have demonstrated activity and potency of siRNAs against the dystrophy protein kinase (DMPK) gene in muscle cells derived from patients with DM1. These data showed significant reductions in DMPK mRNA levels in the nucleus and in the cytoplasm. Treatment with Avidity's therapeutic agent were able to correct the incorrect splicing in these DM1 cells, moving the splicing signature closer to that of healthy cells and indicating the potential to target the underlying cause of DM1. In addition, Avidity scientists have shown the robust, durable activity of AOC 1001 *in vivo* in a broad range of muscles of non-human primates including cardiac muscle. These data demonstrated a 75% reduction in DMPK mRNA levels in muscles following a single dose of AOC 1001.

Avidity's abstract is available on the [AAN meeting website](#). Following regulatory clearance, the company plans to begin a Phase 1/2 study for AOC 1001 in the second half of 2021.

### About Avidity

Avidity Biosciences, Inc. is driven to change lives with a new class of therapies called Antibody Oligonucleotide Conjugates (AOCs) that are designed to overcome current limitations of oligonucleotide therapies in order to treat a wide range of serious diseases. Avidity's proprietary AOC platform combines the tissue selectivity of monoclonal antibodies and the precision of oligonucleotide therapies to access previously undruggable tissue and cell types and more effectively target underlying genetic drivers of diseases. Avidity's lead product candidate, AOC 1001, is designed to treat myotonic dystrophy type 1, and its other muscle programs are focused on the treatment of Duchenne muscular dystrophy, facioscapulohumeral muscular dystrophy, Pompe disease and muscle atrophy. In addition to its muscle franchise, Avidity has research efforts focused on immune, cardiac and other cell types.

Avidity is headquartered in La Jolla, CA. For more information about Avidity's science, pipeline and people, please visit [www.aviditybiosciences.com](http://www.aviditybiosciences.com) and engage with Avidity on [LinkedIn](#).

### 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 current beliefs and expectations. Such forward-looking statements include, but are not limited to, statements regarding: Avidity's evolution to a clinical-stage company; Avidity's plans to conduct clinical trials of AOC 1001 in patients with DM1 and the expected timing thereof; 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: the company is early in development efforts and all of its development programs are currently in the preclinical or discovery stage; the company's approach to the discovery and development of product candidates based on the 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; disruption to the company's operations from the COVID-19 pandemic; the success of its preclinical studies and clinical trials for its product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; its dependence on third parties in connection with preclinical testing and product manufacturing; 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; 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; risks related to integration of new management personnel; and other risks described in the company's prior press releases and in its 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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<https://aviditybiosciences.investorroom.com/2021-04-16-Avidity-Biosciences-to-Present-Oral-Presentation-on-AOC-1001-for-DM1-at-American-Academy-of-Neurology-AAN-2021-Virtual-Annual-Meeting>