Avidity Biosciences Announces 2021 Pipeline Updates and Research Collaboration with MyoKardia

AOC 1001 for DM1 on track for initiation of Phase 1/2 study in 2H 2021
Meaningful pipeline advancement; moving up FSHD clinical trial initiation to 2022 and progressing 3 programs for DMD
Further platform expansion beyond skeletal muscle through research collaboration with MyoKardia, a Wholly-Owned Subsidiary of Bristol Myers Squibb

LA JOLLA, Calif., Jan. 8, 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 pipeline updates for 2021 and a research collaboration with MyoKardia, Inc., a wholly-owned subsidiary of Bristol Myers Squibb.

"In 2021, we enter an important new phase for Avidity as AOC 1001 is expected to enter Phase 1/2 studies for the treatment of adults with myotonic dystrophy type 1, a rare, progressive muscle disease with no approved therapy," said Sarah Boyce, President and Chief Executive Officer. "This milestone is the culmination of years of engineering and research laying the groundwork for the progress of our muscle franchise and future expansion of our AOC platform."

2021 Pipeline Updates

- In 2H 2021, Avidity plans to initiate a Phase 1/2 clinical study of AOC 1001 in adults with myotonic dystrophy type 1 (DM1).
- AOC FSHD is a therapeutic program in development for the treatment of facioscapulohumeral muscular dystrophy (FSHD). In 2021, Avidity plans to advance the program into IND-enabling studies. In 2022, Avidity plans to submit a regulatory filing to support a clinical trial.
- AOC DMD is a therapeutic program in development for the treatment of Duchenne muscular dystrophy (DMD). Avidity has expanded its efforts for this indication and is now advancing three programs for DMD which target different mutations that are amenable to skipping, including Exon 44, Exon 51 and Exon 45. Avidity's lead AOC in development for DMD targets Exon 44. In 2022, Avidity plans to submit a regulatory filing to support a clinical trial.
- AOC Muscle Atrophy is a therapeutic program in development for the treatment of muscle atrophy (MA). During 2021, Avidity researchers will evaluate AOCs in multiple disease models of MA to identify an optimal development path.

Research Collaboration with MyoKardia, a Wholly-Owned Subsidiary of Bristol Myers Squibb

- Avidity has entered into a research collaboration with MyoKardia, a wholly-owned subsidiary of Bristol Myers Squibb, to demonstrate the potential utility of AOCs in cardiac tissue by leveraging MyoKardia's genetic cardiomyopathy platform including, among other aspects, its novel target discovery engine and proprietary cardiac disease models.

"We are excited to partner with MyoKardia, the pioneers in precision medicine approaches to treating cardiomyopathies and other heart diseases," said Joseph Baroldi, Chief Operating Officer. "This research collaboration with MyoKardia furthers Avidity's strategic approach to broadening the use of our AOC platform beyond skeletal muscle. Looking ahead, our business development efforts will continue to focus on collaborating with high-quality partners to accelerate and expand the utility of our AOCs."

About Avidity Biosciences

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 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 our current beliefs and expectations. Such forward-looking

statements include, but are not limited to, statements regarding: the potential to develop a meaningful pipeline of novel AOC therapeutics; the initiation of a Phase 1/2 clinical trial of AOC 1001 in patients with DM1 and the timing thereof; Avidity's plans to advance the AOC FSHD program into IND-enabling studies and submit a regulatory filing to support a clinical trial and the expected timing thereof; the advancement of three programs for DMD and potential development for AOC MA; the potential to identify new targets beyond the muscle that can be targeted with Avidity's AOC approach, including cardiac tissue under the MyoKardia collaboration; the future expansion of Avidity's AOC platform; and the broad potential of AOCs to treat serious diseases. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of our plans will be achieved. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in our business, including, without limitation: we are early in our development efforts and all of our development programs are in the preclinical or discovery stage; our approach to the discovery and development of product candidates based on our AOC platform is unproven, and we do not know whether we will be able to develop any products of commercial value; potential delays in the commencement, enrollment and completion of clinical trials; disruption to our operations from the COVID-19 pandemic; the success of our preclinical studies and clinical trials for our product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; we may not be able to show utility of our AOCs in cardiac or other tissue and we may not realize any benefits from the MyoKardia collaboration; our dependence on third parties in connection with preclinical testing and product manufacturing; unexpected adverse side effects or inadequate efficacy of our 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 our proposed design of future clinical trials; risks related to integration of new management personnel; and other risks described in our prior press releases and in our 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 we undertake 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.

Contacts:

Company:

Mike MacLean
(858) 401-7900
mikemaclean@aviditybio.com

Media and Investors:

Amy Conrad
Juniper Point
(858) 366-3243
amy@juniper-point.com

SOURCE Avidity Biosciences, Inc.
