ARROWHEAD PHARMACEUTICALS

Fiscal 2023 Third Quarter Conference Call – Prepared Remarks

August 7, 2023

1:30 PM Pacific time

Operator

Ladies and gentlemen welcome to the Arrowhead Pharmaceuticals conference call. Throughout today's recorded presentation all participants will be in a listen-only mode. After the presentation, there will be an opportunity to ask questions. I will now hand the conference call over to Vince Anzalone, Vice President of Investor Relations for Arrowhead. Please go-ahead Vince.

Vince Anzalone

Good afternoon and thank you for joining us today to discuss Arrowhead's results for its fiscal 2023 third quarter ended June 30, 2023.

With us today from management are president and CEO Dr. Chris Anzalone, who will provide an overview of the quarter; Dr. Javier San Martin, our chief medical officer, who will provide an update on our mid and later stage clinical pipeline; Dr. James Hamilton, our Chief of Discovery & Translational Medicine, who will provide an update on our earlier stage programs; and Ken Myszkowski, our chief financial officer, who will give a review of the financials. In addition, Tracie Oliver, our chief commercial officer, and Patrick O'Brien, our chief operating officer and general counsel, will be available during the Q&A portion of the call.

Before we begin, I would like to remind you that comments made during today's call contain certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. All statements other than statements of historical fact are forward-looking statements and are subject to numerous risks and uncertainties that could cause actual results to differ materially from those expressed in any forward-looking statements. For further details concerning these risks and uncertainties, please refer to our SEC filings, including our most recent annual report on Form 10-K and our quarterly reports on Form 10-Q.

I'd now like to turn the call over to Christopher Anzalone, President and CEO of the Company. Chris?

Chris Anzalone

Thanks Vince. Good afternoon everyone and thank you for joining us today.

Our industry is built on promise. Sometimes this promise can be stunning and carry with it the possibility of saving the lives of some and drastically improving life for others. Arrowhead's mission is to bring important new medicines to the people who need them and save lives and alleviate suffering where we can. While this is our guiding principle and focusing on this promise has given us purpose and the motivation to, I believe, innovate at industry-leading levels and operate at speeds not seen before, it is not the only important focus for us. Another is risk.

Our industry swims in a sea of risk. We recognize that in order to succeed, we need to appreciate the great promise in front of us, but focus on all the risk between

idea and the medicine ultimately given to a patient. We are idealists, but we are not naïve: one of our most important jobs is to mitigate and decrease risk where we can. We have made great progress on this broad front since our last call, and this is how I would like to frame our discussion today.

Let's begin with pulmonary. We believe we've taken an important step toward further de-risking the entire pulmonary franchise with the first chronic GLP toxicology results starting to come in. For ARO-MMP7 the NOAEL, or No Observed Adverse Effect Level, was the highest dose we tested in our chronic rat study. In other words even at the highest dose tested, we were not seeing anything that is deemed adverse. The highest dose represents what we believe would be substantially greater exposure than would be applied to humans. We are waiting for final rat data from the ARO-RAGE chronic GLP tox study, but that also is looking like the NOAEL will be the highest dose we tested. We are still waiting for the 9-month monkey data in both candidates, but our experience with ARO-ENAC leads us to believe that the rat is the more sensitive species for these pulmonary tox studies, so it is very encouraging to us that the rodent studies look so positive.

This is potentially a big step forward for the platform. As you may recall, we saw lung inflammation in some of the chronic GLP tox doses for ARO-ENAC in 2021. Based on our analysis of those results, we concluded that we needed to increase the potency of our pulmonary candidates, and we clearly did that with ARO-MMP7, ARO-RAGE, and ARO-MUC5AC. We were optimistic that these improvements would translate into better chronic tox results, but of course we couldn't know until the data came in. As we are now seeing preliminary data from those studies come in, we are increasingly confident that ARO-MMP7 and likely ARO-RAGE may have substantially wider tox windows than ARO-ENAC did and, I believe, this

represents a significant de-risking event for the pulmonary franchise. We look forward to having complete rat and monkey chronic GLP tox data for ARO-RAGE and ARO-MMP7 in coming months and expect to have chronic GLP tox data for ARO-MUC5AC next year.

Encouraging preliminary chronic GLP tox data follow prior de-risking events in the pulmonary franchise over the past quarter. Specifically, I believe the ARO-RAGE clinical data indicate three important things:

First, the safety and tolerability reports to date have been good and nothing surprising has emerged. This is always a critical first step for a new platform and for every new drug.

Second, the activity data have been impressive and showed a continued dose response through the top dose level. After just a single inhaled dose of 184 mg ARO-RAGE, we saw up to 95% knockdown with a mean of 90% in that cohort. Not only is this a high level of target gene knockdown, but it was extraordinarily consistent across participants in the cohort. Each subject had a good response. This is in the same ballpark as what we now expect with optimized liver targeted programs, and this is an important point. I think it is generally accepted that RNAi is a reliable modality to safely reduce expression of a target gene and that when Arrowhead introduces a new liver program, there is a high expectation (both internally and externally) that the drug candidate will reduce expression of the target protein as designed. I am hopeful that with each new data set we are approaching this expectation in pulmonary. That is a giant leap forward and an important value inflection point.

Lastly, we think the data also showed that the duration of effect with ARO-RAGE supports a dosing interval of 2 months or more. This is an important de-risking event because it limits accumulated drug exposure, increasing our confidence that the good safety profile seen thus far may continue during chronic treatment. It would also be a very patient-friendly dosing regimen.

De-risking the pulmonary platform is important for its own sake: as we have said in the past, we see many potential drugs coming out of the franchise that could address a number of unmet medical needs, and we appear to be the only company able to effectively use RNAi in the lungs. The pulmonary franchise alone could be the basis of a large company. But it's also important as an example of how we seek to de-risk our broader business. From our perspective, a 1- or 2-drug company is a bet, not a business. From the beginning, we have sought to create a broadly diversified business to increase the number of patients we serve, but also, importantly, as a hedge against the unpredictability of biology. In our industry, the risk of failure is substantial, and our mitigation strategy has been part innovation and part brute force. We have sought to create a technological platform that works reliably, and then move as fast as we can to create as many well thought out drug candidates as possible.

We've built and continue to refine and expand the reach of our TRiM™ platform. This is a modular, structurally simple system to:

1. Address multiple cell types, which allows our therapies to go where a disease is in a way that other RNA companies do not;
2. Move rapidly from idea to the clinic and then efficiently through mid and late stage clinical studies; and

3. Provide platform continuity and confidence, which gives us an enhanced expectation of success for new candidates that we believe far exceeds that of biotech broadly. Lessons learned developing each candidate informs the development of future candidates, so our expectation of success grows stronger over time. We believe this translates to the potential for more candidates to become approved therapies than industry average.

Our "20 in 25" initiative follows this platform development and represents, to some extent, the brute force component of our broader risk mitigation strategy. We have platforms that appear to work well, so we have the responsibility to our patients and stake holders to build as many new drugs as fast as we can. It is our goal to have 20 clinical stage or marketed products by the year 2025.

Somewhat paradoxically, building such a large pipeline is part of our strategy to mitigate balance sheet risk. We are in a very expensive business and one could argue that the best way to ensure we are properly capitalized to bring drugs to market is to have a small focused pipeline. We reject that. Rather, we believe that well thought out drug candidates with greater than industry average chances of success can always find homes in partner companies' pipelines. As we mentioned at our analyst day in June, we have brought in nearly $1bn in partnering capital over the past 6 years and have not raised equity capital for over 3 ½ years. In fact, GSK recently initiated a Phase 2b study of GSK4532990, formerly called ARO-HSD, for the treatment of NASH, which earned us a $30 million milestone payment. In addition, Takeda initiated the Phase 3 REDWOOD study of fazirsiran, being developed as a potential treatment for alpha-1 antitrypsin deficiency liver disease, which earned Arrowhead a $40 million milestone payment.

We believe that partnering is a good cornerstone of a broader financing strategy, and one that our platforms are uniquely suited to because of the quality of the candidates coming out of them and the scarcity of companies that are skilled at generating RNAi-based therapeutics.

Our partnering strategy includes:

- existing partnerships that are maturing and therefore eliciting higher payments;
- new potential partnerships that could combine our platforms with a partner's target or set of targets; and
- new partnerships on existing programs in our pipeline.

Regarding the latter, on our last earnings call I discussed that at that time we had paused the CTA filing because of some inbound interest in partnering ARO-DUX4. We continue to explore those options, however we decided to move forward with the ARO-DUX4 CTA filing ourselves. Partnering discussions can take time and we don't ultimately know if they will translate into license agreements. We felt it did not make sense to further delay the CTA filing and the Phase 1 study.

While partnering continues to be a cornerstone of our financing model, we are certainly cognizant of the risk of over-partnering. We believe the best way to build a lot of value quickly is to retain some wholly-owned candidates and drive toward commercialization. Of course there is substantial risk with this course, but over the past quarter we believe we have taken some off the table.

We completed enrollment in the Phase 3 PALISADE study of ARO-APOC3 in patients with familial chylomicronemia syndrome, or FCS. This is an important

milestone for Arrowhead because it will likely be the first candidate and indication that we will seek regulatory approval for. The final study visit for the last patient in is scheduled for Q2 2024, so we expect to start the NDA process next year. In addition to FCS, we are currently working on the Phase 3 plans for severe hypertriglyceridemia and mixed dyslipidemia, which we will be discussing with regulators this year. Shortly after those discussions we plan to start Phase 3 studies for those larger indications.

Our other wholly-owned cardiometabolic candidate, ARO-ANG3, also had an important milestone during the quarter. We presented data at the European Atherosclerosis Society Congress demonstrating that ARO-ANG3 achieved LDL-C reductions of 44% to 48% when added to existing standard-of-care treatments. These results are similar to results seen in studies of an approved monoclonal antibody targeting ANGPTL3 in patients with HoFH. These are important de-risking data as we move toward one or more Phase 3 programs, which we are currently designing.

We are actively working on go to market strategies for multiple candidates. We expect to have 4 drug candidates in Phase 3 studies by the end of the year. Two of these are currently wholly-owned, ARO-APOC3 and ARO-ANG3, and a third, fazirsiran, is partnered with a 50-50 profit share in the US so we have retained substantial economics. As I mentioned, we will have our first Phase 3 registrational study readout mid next year for our ARO-APOC3 program in FCS and expect an NDA soon thereafter. As we look at our pipeline, we would expect additional NDA filing opportunities on a very regular basis going forward.

Moving to our earlier-stage pipeline, we filed two CTAs for two new programs targeting gene expression in two different tissue types. I already mentioned ARO-

DUX4 in skeletal muscle for treatment of FSHD, and the other is ARO-SOD1 in the central nervous system for the treatment of ALS. We expect additional CTAs over the next few quarters using both the CNS and skeletal muscle platforms. Of course these are early, but they represent important de-risking events for potential CNS and skeletal muscle franchises. As with our advances in pulmonary, these are illustrative of our desire to expand the reach of our technology and decrease the overall risk of our business by creating value across many different channels.

Lastly, before handing the call over to Javier, I want to highlight the R&D Day that we hosted in June. During that presentation, which is still available to view on our website, we gave updates and had external KOLs talk about some of existing clinical programs in cardiometabolic and pulmonary diseases and discussed what's next for us in CNS tissue, including potentially systemic delivery, and delivery to adipose tissue. The R&D Day had a lot of detail. We are constantly pushing our technology forward and expanding its reach.

With that overview, I'd now like to turn the call over to Dr. Javier San Martin. Javier?

Javier San Martin

Thank you, Chris, and Good Afternoon everyone.

The design, planning, and preparation of the late-stage studies of our cardiometabolic candidates, ARO-APOC3 and ARO-ANG3, is well under way. We are making good progress towards our goal of conducting multiple end of Phase 2 meetings with regulators this year and initiating multiple Phase 3 studies late this year and early next year. We also intend to presentfinal Phase 2 clinical

data at the American Heart meeting in November, pending abstract acceptance, for multiple studies of both ARO-APOC3 and ARO-ANG3.

Let's take a moment to review the various studies we have conducted and then I will provide our current thinking around what the Phase 3 studies may look like for each clinical indication.

I will start with ARO-APOC3, our investigational RNAi therapeutic being developed as a treatment for patients with mixed dyslipidemia, severe hypertriglyceridemia, and familial chylomicronemia syndrome. ARO-APOC3 is designed to reduce production of Apolipoprotein C-III, or APOC3, a component of triglyceride rich lipoproteins including very low-density lipoproteins, or "VLDL." and chylomicrons and is a key regulator of triglyceride metabolism. Knocking down the hepatic production of APOC3 by RNAi results in reduced VLDL synthesis and assembly, enhanced breakdown of triglyceride rich lipoproteins, and better clearance of VLDL and chylomicron remnants via LPL dependent and independent pathways. We view ARO-APOC3 as having the potential to address many patient populations with various lipid disorders that can lead to different clinical complications and phenotypes.

Familial chylomicronemia syndrome, or FCS, is characterized by extremely high TG levels, typically over 1,000 mg/dL and as high as 5,000mg/dL, leading to high risk of acute pancreatitis that usually requires hospitalization and can be fatal. Patients with FCS may also experience chronic abdominal pain and they have to adhere to a very strict diet with very low fat content leading to impair the quality of life. FCS is a severe and ultrarare genetic disease that affect hundreds to a few thousand patients in the U.S.

Severe hypertriglyceridemia, or SHTG, is characterized by marked elevations in TG levels, typically over 500 mg/dL, which can lead to increased risk of acute pancreatitis, as well as an increased risk of cardiovascular disease. This condition is estimated to affect several million patients in the U.S.

Lastly, Mixed dyslipidemia is defined as the presence of high LDL-Cholesterol combined with high TGs, Remnant cholesterol and low HDL. This lipid profile is a major component of the risk factor for atherosclerotic cardiovascular disease. There are likely tens of millions of patients in the U.S. with mixed dyslipidemia who are not adequately controlled with current standard-of-care.

The studies of ARO-APOC3 that we have conducted or are planning to conduct for each population are as follows:

For FCS, we are conducting the PALISADE study, which is a Phase 3 placebo-controlled study to evaluate the efficacy and safety of ARO-APOC3 in adults with FCS. The primary endpoint of the study is percent change from baseline in fasting TG at Month 10. This study was fully enrolled in May with a total of 75 subjects distributed across 39 different sites in 18 countries who were randomized to receive 25 mg ARO-APOC3, 50 mg ARO-APOC3, or matching placebo once every three months. This puts us on schedule for study completion in Q2 of 2024, a data readout shortly thereafter, and then NDA preparation for regulatory filling. Participants who complete the randomized portion of Palisade are also eligible to continue in an extension period, where all participants will receive ARO-APOC3.

For SHTG, we are conducting the SHASTA-2 Phase 2 study in 229 patients randomized 3:1 to receive 10, 25, or 50 mg ARO-APOC3 or placebo on day 1 and at week 12. Patients with FCS were excluded from this study. The primary

endpoint is percent change from baseline in fasting TGs at week 24. SHASTA-2 was the study that enabled us to begin planning and design for our Phase 3 plan in SHTG patients. The data strongly support advancement into Phase 3 and we plan to present results at AHA in November.

The current Phase 3 plan for SHTG includes two separate studies, which will be called SHASTA-3 and SHASTA-4. The idea behind the two studies is to have first, a faster path to regulatory submission with the SHASTA-3 study, a double blind 12 months, randomized control study of approximately 600 patients with TG>500mg/dL, we believe this study, plus the large safety database from our phase 1 and 2 studies will be an appropriate package for the initial filing for the sHTG indication. The second study SHASTA-4 is designed to investigate the effect of ARO-APOC3 in a more severe population at high risk of developing pancreatitis. SHASTA-4 will include patients with TGs>880mg/dL and recent history of pancreatitis, the duration of the double-blind portion of the study will be 2 years and it will be power to detect difference in the incidence of pancreatitis. These data could enable label expansion to include the indication statement of pancreatitis risk reduction, a key clinical outcome relevant to patients and reimbursement authorities. We have made a lot of progress on the planning and design for these studies and intend to have discussions with regulators this year and move forward rapidly with study initiation. We will provide more details on the studies when they begin.

In the broader mixed dyslipidemia population, we are conducting the MUIR Phase 2 study in 353 patients randomized 3:1 to receive 10, 25, or 50 mg ARO-APOC3 or placebo on day 1 and at week 12. We include an additional cohort of participants receiving 50 mg on day 1 and week 24. The primary endpoint is percent change from baseline in fasting TGs at week 24, with additional

assessments of the changes in various other lipid parameters such as LDL-C, Non-HDL-C, HDL-C, ApoB, , VLDL-C, and other biomarkers. Similar to the SHTG study results, we believe the Phase 2 data from the MUIR study strongly support advancement into a Phase 3 study and we also plan to present these results at AHA.

The Phase 3 program for this population will be a cardiovascular outcomes trial called CASCADE. ARO-APOC3 has demonstrated a positive effect on several lipid parameters that represent residual risk factors for atherosclerotic cardiovascular disease even after LDL is well-controlled. The CASCADE study, will select the patient population with high risk driven by the high TGs, remnant cholesterol and low HDL, all of which are effectively addressed by ARO-APOC3. The study will be designed in collaboration with an Academic Research Organization, or "ARO"). We are working on all aspects of the study design including, selection of the patient population, understanding and modeling background event rates, the potential effect size and with that information we'll define the sample size and duration of the exposure to be able to detect a clinically meaningful reduction in cardiovascular events. We are finalizing agreements with the selected CRO and ARO to help us conduct this important study. We are on schedule to engage with regulators later this year and plan to initiate the CASCADE study in 2024.

Our strategy for ARO-APOC3 is to progressively study it in larger and longer studies to potentially bring it to very high prevalence disease populations that currently do not have adequate treatment options. Our strategy for ARO-ANG3 is more focused on a smaller well-defined population. ARO-ANG3 is being developed as a treatment for homozygous familial hypercholesterolemia, or HoFH,

and potentially in the future subsets of heterozygous familial hypercholesterolemia, or HeFH.

The Phase 2 program for ARO-ANG3 involved two studies: the ARCHES-2 Phase 2 study in 204 patients with mixed dyslipidemia; and the GATEWAY study in 18 patients with HoFH. Interim data for the GATEWAY study was presented at the 91st European Atherosclerosis Society Congress in May 2023. At study week 20, administration of 200 mg or 300 mg ARO-ANG3 on day 1 and day 84 led to mean reductions in LDL-C of 48.1% and 44.0%, respectively. These reductions were achieved on top of continued standard of care, including statins, ezetimibe, PCSK9 inhibitors, and apheresis. These results were on par with an approved monoclonal antibody that also targets ANGPTL3, ARO-ANG3 has a much more convenient and patient-friendly dosing regimen of one subcutaneous injection every 3 months versus the antibody which requires an intravenous infusion once a month. We are currently working on a Phase 3 study design and plan for ARO-ANG3 in HofH and assessing potential other patient populations for future study.

I spoke in a bit more detail on both ARO-APOC3 and ARO-ANG3 during our R&D Day in June. I recommend you view the archived webcast or presentation slides on our website if you want more background on the biology of the targets, some of the clinical data, the rationale for our belief in their potential, and more specific info about our plans for clinical development.

The other late-stage program we are working on with our partner Takeda is fazirsiran for the treatment of AATD liver disease. In June, updated Phase 2 clinical data from the SEQUOIA study were presented at EASL Congress 2023 in an oral presentation. The clinical results from the Phase 2 SEQUOIA study of

fazirsiran were clear and compelling. Fazirsiran treatment demonstrated a substantial effect on several key markers of liver disease.

Takeda has taken the lead in conducting the Phase 3 REDWOOD clinical study. It is designed to enroll 160 adult patients with F2 to F4 fibrosis. The primary endpoint of the study is a decrease from baseline of at least 1 stage at Week 106 in patients with F2 and F3 fibrosis. Takeda is doing an outstanding job at bringing global sites online for the REDWOOD study and enrolling patients efficiently. Additional information on the REDWOOD study can be found at www.theredwoodliverstudy.com.

I will now turn the call over to Dr. James Hamilton. James?

James Hamilton

Thank you, Javier.

Our pipeline of early-stage clinical candidates now includes 8 programs addressing various diseases with gene expression in four tissue types: liver, lung, and now muscle and CNS. Of these 8 programs most are wholly-owned and in our core areas of focus. They are:

- In pulmonary: ARO-RAGE, ARO-MUC5AC, and ARO-MMP7
- In cardiometabolic: ARO-PNPLA3
- In neuromuscular: ARO-DUX4 and ARO-SOD1
- We also have ARO-C3 for complement mediated diseases and HZN-457, partnered with Horizon, for gout

In addition, we have many undisclosed preclinical programs that should continue to feed our pipeline for years to come. We are increasingly looking for opportunities to focus around core areas, and we are fortunate that our platform provides us with so many opportunities.

Our discovery and clinical development teams continue to be highly productive and efficient. One main benefit of drug development based on a proprietary technology platform is that it allows us to apply learnings from prior programs to each new program. This makes us faster, more precise, and I believe yields drug candidates with a higher probability of success.

The TRiM™ platform has given us that advantage for liver directed programs for a few years now. We believe we are now in a period where those same advantages exist for lung directed programs, and we have the potential to get there over the next couple years for muscle and CNS.

We held a very comprehensive R&D Day during the quarter, so I'm not going to review all Arrowhead's discovery and early development programs. I'd like to focus on some important potentially de-risking data from our ARO-RAGE program.

ARO-RAGE is our RNAi therapeutic candidate designed to reduce expression of the receptor for advanced glycation end products, or RAGE, as a potential treatment for inflammatory pulmonary diseases, such as asthma. We are currently conducting a Phase 1/2a clinical trial in normal healthy volunteers and in patients with mild-to-moderate asthma. We have also recently filed an amendment to add a cohort of asthma patients with high baseline levels of fractional exhaled nitric

oxide, or FeNO, which is a biomarker for the degree of IL-13 driven type 2 inflammation in the lung.

Let's talk briefly about what data we generated and reported at the R&D day.

First, with respect to safety and tolerability, to date there have been no reported serious or severe adverse events, no study withdrawals or drug discontinuations due to adverse events, and safety labs have shown no patterns of adverse changes. There has also been no change in the pattern of airway immune cells and all chest X-rays were read as normal. These encouraging results have also been generally consistent in the ARO-MMP7 and ARO-MUC5AC programs.

With respect to activity, the results to date, especially at the highest dose level, have exceeded our estimates and really represent a best-case scenario for target engagement. We are measuring soluble RAGE protein, or sRAGE, in serum after multiple doses in both healthy volunteers and patients, and in BALF after a single dose in healthy volunteers and after multiple doses at the top dose level.

The mean maximum reduction in sRAGE, at the 92 mg dose level after two doses on Day 1 and Day 29 was 80% with a maximum reduction of 90%, with a long duration of effect that supports every other month dosing. At the highest dose of 184 mg, we achieved a similar result after just a single dose, with mean sRAGE reduction of up to 76% with maximal reduction of 91%. We also observed a continued dose response in BALF with a single inhaled dose of 184 mg achieving mean reduction of 90% and maximal reduction of 95%. We are still collecting data that we intend to report on later this year, including presentations at the European Respiratory Society International Congress in September.

We believe this is the first compelling clinical evidence of gene target silencing in the lung using siRNA. We also believe that these clinical results have a good chance of being predictive of clinical results in other pulmonary programs, including ARO-MUC5AC and ARO-MMP7, and additional undisclosed preclinical programs.

And lastly on RAGE, what data are we generating over the coming months?

We will have the chronic monkey GLP toxicology results before the end of the year, which will be needed prior to Phase 2 initiation. We will be getting additional longer term follow up and multiple dose data at the highest dose in healthy volunteers and patients later this year and into next year. Lastly, we will be getting data from the high FeNO cohorts, which is designed to assess if RAGE knockdown leads to an IL-13 specific anti-inflammatory effect. This study is not long enough or large enough to expect an efficacy signal, but signs of inflammatory pathway inhibition after short exposure would be a welcome result. We expect these data in 2024.

I also want to provide an update on our earliest clinical candidates. During the last quarter we filed CTAs for our first muscle and CNS candidates, ARO-DUX4 and ARO-SOD1 respectively.

ARO-DUX4 is the first clinical candidate utilizing the TRiM^{TM} platform to target disease associated genes in skeletal muscle. ARO-DUX4 is an investigational RNAi therapeutic designed to reduce expression of the gene that encodes the human double homeobox 4, or DUX4, protein as a potential treatment for facioscapulohumeral muscular dystrophy, or FSHD.

Pending regulatory clearance, we intend to proceed with a Phase 1/2a dose-escalating study to evaluate ARO-DUX4 in adult patients with FSHD type 1. The study is designed to enroll up to 52 patients.

The other CTA filed during the quarter was for ARO-SOD1, the first therapeutic candidate designed for delivery to the CNS, again leveraging the TRiM™ platform. ARO-SOD1 is designed to reduce expression of superoxide dismutase 1, or SOD1, in CNS as a potential treatment for patients with amyotrophic lateral sclerosis, or ALS, caused by SOD1 mutations.

Pending regulatory clearance, we intend to proceed with a Phase 1 dose escalation study to evaluate ARO-SOD1 in adult patients with ALS harboring a SOD1 mutation, which is considered to be causative of ALS. The study is designed to enroll up to 24 patients.

I will now turn the call over to Ken Myszkowski. Ken?

Ken Myszkowski

Thank you, James, and good afternoon, everyone.

As we reported today, our net loss for the quarter ended June 30, 2023 was $102.9 million or $0.96 per share based on 107.0 million fully-diluted weighted average shares outstanding. This compares with net loss of $72.0 million or $0.68 per share based on 105.8 million fully-diluted weighted average shares outstanding, for the quarter ended June 30, 2022.

Revenue for the quarter ended June 30, 2023 was $15.8 million, compared to $32.4 million for the quarter ended June 30, 2022. Revenue in the current period primarily relates to our collaboration agreement with Takeda.

Revenue is recognized as we complete our performance obligations, which include managing the ongoing AAT Phase 2 clinical trials for Takeda. There remains $17 million of revenue to be recognized associated with the Takeda collaboration which we anticipate will be recognized over the next year.

Total operating expenses for the quarter ended June 30, 2023 were $118.5 million, compared to $105.3 million for the quarter ended June 30, 2022. The key drivers of this change were increased candidate costs, partially offset by lower stock compensation expense. The increased candidate costs were primarily due to the progression of the Company's pipeline of candidates into and through clinical trials, which resulted in higher outsourced clinical trial, toxicity study and manufacturing costs.

Net cash used in operating activities during the three months ended June 30, 2023 was $21.4 million, compared with net cash used in operating activities of $68.9 million for the three months ended June 30, 2022. We expect our operating cash burn to be $80 to $90 million next quarter. We expect to spend between $160 million and $180 million over the next three quarters to complete our GMP manufacturing facility and related laboratories in Verona.

Turning to our balance sheet, our cash and investments totaled 494.5 million at June 30, 2022, compared to $482.3 million at September 30, 2022. The increase in our cash and investments was primarily related to the $250.0 million payment from

Royalty Pharma, as well as other licensing cash inflows, offset by our operating cash burn along with continuing capital projects.

Our common shares outstanding at June 30, 2023, were 107.1 million.

With that brief overview, I will now turn the call back to Chris.

Chris Anzalone

Thanks Ken.

We are well on our way to reaching our "20 in 25" goal to grow our pipeline of RNAi therapeutics to a total of 20 clinical stage or marketed products in the year 2025. However, pipeline expansion is just a means to an end. The ultimate goal and the reason we continue to invest in expanding our platform, discovering new candidates, advancing our clinical programs, and streamlining the drug manufacturing process is that it allows us to get important new medicines to patients in need as quickly and efficiently as possible. Doing this will also create a sustainable business and provide a steady stream of commercial revenue, which we now have a better line of sight on and plan that we are executing on to get there.

Thank you for joining us today and I would now like to open the call to your questions. Operator?

Operator