ARROWHEAD PHARMACEUTICALS

Fiscal 2020 Second Quarter Conference Call – Prepared Remarks

May 7, 2020

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 Vincent Anzalone, Vice President of Investor Relations for Arrowhead. Please go-ahead Vince.

Vince Anzalone

Good afternoon everyone. Thank you for joining us today to discuss Arrowhead's results for its fiscal second quarter ended March 31, 2020.

With us today from management are president and CEO Dr. Christopher Anzalone, who will provide an overview of the quarter; Dr. Javier San Martin, chief medical officer, who will discuss our clinical programs; Dr. Curt Bradshaw, our chief scientific officer, who will discuss our discovery, platform development, and manufacturing efforts; and Ken Myszkowski, our chief financial officer, who will give a review of the financials. In addition, James Hassard, our chief commercial officer will be available during the Q&A session of today's call.

This is the first earnings call without Dr. Bruce Given, who retired last week, since he joined the company 10 years ago. I want to start by thanking Bruce for all of his contributions to Arrowhead. Bruce developed strong relationships with the investment community over the years, and I am certain that Javier, Curt, and Jim will do the same.

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 27(A) of the Securities Act of 1933 and Section 21(E) of the Securities Exchange Act of 1934. All statements other than statements of historical fact, including without limitation those with respect to Arrowhead's goals, plans, and strategies are forward-looking statements. These include statements regarding our expectations around the development, safety and efficacy of our drug candidates, projected cash runway, and expected future development and commercialization activities. These statements represent management's current expectations and are inherently uncertain. Thus, actual results may differ materially. Arrowhead disclaims any intent and undertakes no duty to update any of the forward-looking statements discussed on today's call.

You should refer to the discussions under risk factors in Arrowhead's annual report on Form 10-K and the Company's subsequent quarterly reports on Form 10-Q for additional matters to be considered in this regard, including risks and other considerations that could cause actual results to vary from the presently expected results expressed in today's call.

With that said, I'd 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.

I would like to start by thanking Dr. Bruce Given. He did a fantastic job developing the R&D organization and instilling a culture of innovation that will be a lasting legacy. Bruce's contributions are clear to all who know him or have worked with him, and I'm confident that we have assembled and developed the right team to enable us to continue to execute in Arrowhead fashion. Javier, Curt, and Jim are seasoned executives who are expanding and professionalizing their respective functions and have put Arrowhead in a very strong position to prosper during this next phase of growth.

2020 is the year that we intend to make the promise of RNAi outside the liver a reality. We hope to gain clinical proof-of-concept in the lung and solid tumors and then rapidly expand our pipeline, much as we did with our hepatocyte targeted pipeline in 2018 and 2019. This could provide new treatment options for countless patients and provide Arrowhead shareholders with continued value growth.

While of course we're looking to expand our reach, we are also looking to squeeze as much risk out of our business as we can. We have made good progress on that front as well:

- First, we are well capitalized;
- Second, we are developing an increasingly validated platform in TRiM™. We've treated a total of 273 human subjects with 570 doses across our clinical programs, and keep in mind that this does not include all the people who have been treated by Janssen and Amgen with our partnered candidates;

- Third, RNAi is an increasingly validated modality;
- Fourth, we are addressing gene targets that experts generally view as well validated;
- Fifth, we are addressing unmet medical needs; and
- Sixth, we are the first RNAi player in all of our clinical programs.

All of this puts us on solid footing, but the elephant risk in the room is COVID-19. This has to be the lens through which you view our business, at least in the near- to mid-term. Therefore, I will use my portion of this call to provide a high-level overview of our programs and how they may be affected by the outbreak.

Let's start with ARO-AAT, our investigation medicine against alpha-1 liver disease. In March, we voluntarily paused new patient screening and enrollment for at least a 4-week period in the Phase 2/3 SEQUOIA study and the AROAAT2002 open label study. We are now working with sites and investigators to begin the process of resuming screening and enrollment. Any patients already enrolled in these studies continue to be dosed per protocol and continue to come in for their follow up visits. Importantly, protocol deviations have not been out of the ordinary. Before the enrollment pause, we were already fully enrolled in the first cohort of the 2002 study, so we are still on schedule to collect 6-month biopsies in the summer. We intend to report those data in the fall at an appropriate venue.

We believe this is an important readout for the program and the field. While we don't expect to see histological changes after that short amount of treatment, it may provide an early indication that the drug is doing what it is designed to do; which is to reduce new production of the mutant, misfolded AAT protein. This will be assessed by measuring the amount of AAT monomer in hepatocytes. Further, it

will be interesting to compare pre- and post-treatment levels of accumulated AAT polymer. This might give us a view of the pace at which hepatocytes can breakdown and clear the polymerized protein, which is the root cause of the progressive liver disease in patients with the homozygous ZZ mutation. We believe these data are important for us and the field, and we expect this readout to be the first of its kind, anywhere. This underlines both our substantial lead in developing a treatment for alpha-1 liver disease and our position as thought leaders in the field.

There are other RNAi based approaches, but to date none have reported data demonstrating tolerability and pharmacologic activity, even in normal healthy volunteers. In addition, there are other approaches to treating AAT deficiency by correcting the mutant AAT protein in hopes of allowing it to be more efficiently exported from hepatocytes. We see some serious challenges for that approach to show clinical benefit for patients with liver manifestations of AAT deficiency.

First, the liver produces an estimated 2 grams of AAT protein per day. We do not believe it is feasible to administer enough small molecule corrector to address that level of protein production, so we think some portion will likely still accumulate in the liver. So, if the goal is to correct 20-30% of protein (which would still require a very large amount of corrector), that would mean 70-80% of the misfolded protein is still present, which is a lot for the liver to handle. Our data suggest that ARO-AAT is nearly completely suppressing liver production of the mutant Z-AAT protein, and we still believe that it may take 2 years of treatment to show a meaningful change in liver histology. So, how long would you have to treat with a corrector that is leaving a large majority of Z-AAT in the liver? Eight years? Ten years? We think that presents a serious challenge in a clinical trial setting and gives us confidence that we are in a strong competitive position.

Let's now talk about the status of our two cardiometabolic candidates, ARO-APOC3 and ARO-ANG3.

ARO-APOC3 is being developed as a potential treatment for patients with severe hypertriglyceridemia and a history or high risk of pancreatitis. Some of these patients have a single genetic cause for their disease, such as familial chylomicronemia syndrome or FCS, but a significantly larger population has polygenic causes for their hypertriglyceridemia. This is called multifactorial chylomicronemia or MCM and we believe there are around 30,000 patients with this condition in the U.S. alone. FCS and MCM have very similar clinical manifestations. They are both severe diseases that can lead to severe abdominal pain, recurrent pancreatitis, emergency room visits and hospitalizations, and even death. These come with a very high cost with respect to both patient quality of life and economic cost to the healthcare system.

We have decided to focus on the MCM population and are working on a plan for a potentially pivotal study in MCM patients. We intend to request a meeting with FDA and EMA this year to discuss some key study design considerations for a registrational study. We designed the Phase1/2 study to provide sufficient data to enable rolling directly into a Phase 3 study. We will know more after we speak with regulators, but our hope is that we can start a pivotal study in the first half of 2021. We believe that should be a relatively short study, so we continue to believe that ARO-APOC3 could be our first marketed product.

We continue to generate data in the Phase 1/2 clinical study. It was nearly fully enrolled prior to the COVID-19 outbreak, and we have experienced a slight delay in accruing the remaining patients. We already have a substantial amount of data

that we intend to present at various times this year and are hopeful that we will be able to present a full data set later this year. With the disruptions to the traditional medical meeting cycle because of COVID-19 it is unclear what form these data releases will take. We are committed to finding alternative ways to present data, if medical meetings continue to be canceled or postponed.

The early data have been exciting and we expect that trend to continue. As we reported on our last conference call, we have seen approximately 95% reduction of circulating triglycerides in hypertriglyceridemic patients after only a single dose of ARO-APOC3. This is truly stunning. These patients have triglycerides in the thousands, so we would expect a reduction of this magnitude to be quite meaningful for patients like these.

We are in a slightly more advanced position with ARO-ANG3, which is being developed as a potential treatment for patients with mixed dyslipidemia. Let's talk about that patient population for moment.

Mixed dyslipidemia patients have both elevated triglycerides and elevated LDL-cholesterol and are at heightened risk for atherosclerotic cardiovascular disease. There is strong evidence that both triglycerides and LDL contribute to that risk. This is a very high-prevalence disease with an estimated potential patient population of between 10-15 million people in the U.S. alone and it is not adequately addressed with current standard-of-care. We see ARO-ANG3 potentially being able to reduce triglycerides to a far higher degree than other available treatments and also reduce LDL in a non-LDL receptor mediated manner, making LDL reduction potentially greater than with statins and PCSK9 inhibitors alone. As with ARO-APOC3, we reported early patient data at our last conference call, and I believe that it was impressive. We saw approximately 80% reduction in

triglycerides and 40% reduction in LDL after only a single dose of ARO-ANG3. Importantly, all of these patients were already on LDL-lowering drugs, such as statins and PCSK9 inhibitors.

The patient population we expect to address is quite large, and a pivotal study to show a reduction in cardiovascular events would also be large. We, and we think most experts in the field, believe strongly that mixed dyslipidemia patients are in need of new treatment options, and we believe the mechanism of ANGPTL3 reduction is very intriguing. So, we are now determining what the regulatory and development path would look like for that indication. Because of our focus on this high-prevalence population, we will likely need to run a Phase 2b study instead of rolling directly into a pivotal study, as we may be able to do with ARO-APOC3. We plan on engaging with FDA this year and hope to initiate the Phase 2b in the first half of 2021.

The ARO-ANG3 Phase 1/2 study is making good progress, even in the COVID-19 environment. The study is fully enrolled, so we do not expect any real delays as we continue to follow patients and generate data. We believe we will have a full dataset to report later in the year and will also look for opportunities to share data subsets throughout the year. Just like ARO-APOC3, we will assess alternative ways to present data, if medical meetings continue to be canceled or postponed.

I would now like to move on to our newest clinical candidates:
- ARO-HIF2 in development to treat the clear cell form of renal cell carcinoma, for which we filed an IND in December;
- ARO-HSD in development to treat alcohol and non-alcohol related liver disease, for which we filed a CTA in December; and,

- ARO-ENaC in development to treat cystic fibrosis, for which we filed a CTA recently.

We expect the ARO-HIF2 Phase 1 study to begin enrolling shortly. We have 1 site open now for screening and enrollment and we anticipate the first patients will be dosed this quarter. The start-up process for this study has taken longer than we hoped, which is likely due in part to COVID-19. Many of the investigators are at academic centers, and the contracting and initiation process may be experiencing delays due to health and safety precautions.

We expect to potentially have proof-of-concept data for the candidate and for the tumor targeted TRiM^{TM} platform this year, but the timing may be too tight to report anything publicly until next year. We should have a better idea about this as we see the pace of enrollment. We are already thinking about additional targets for the tumor program and we intend to build out the pipeline once we have clinical proof of concept, on our own and potentially in collaboration with a partner.

What does success look like for the current Phase 1? We will be taking biopsies from metastases and if we see good Hif2-alpha knockdown, we will be happy that we are on the right track. First, because Hif2-alpha is a well validated target for the approximately 80% of patients with clear cell RCC who have the von hippel lindau mutation, we would have an expectation that ARO-Hif2 could be helpful for these patients. Second, because our targeting strategy is intended to work *across* different solid tumor types rather than just in RCC, Hif2-alpha knockdown would suggest that we might have a broad solid tumor franchise. Once we achieve clinical proof of concept that we are knocking down hif2-alpha, our goal is to quickly expand into new solid tumors against new targets. We view this is a scalable and rapid value creation strategy.

ARO-HSD began dosing in a Phase 1/2 study in March. We are through the first cohort and we previously received approval from the safety monitoring committee to escalate to the next higher dose. Because of COVID-19 related restrictions in New Zealand, enrollment of this second cohort was paused, but we expect it to re-open for healthy volunteers shortly and patients sometime after that. We are working with the site on plans to restart enrollment and believe this is only a minor delay that we don't think will have any lasting effects on the program or our general timelines.

For ARO-ENaC, we filed a CTA last month to begin a Phase 1/2 study in healthy volunteers and in patients with Cystic Fibrosis. It's too early to say if there will be any COVID-19 related delays in this program. That largely depends on what happens in the next couple of months and beyond. Our belief is that we will be able to generate data on the safety and activity of the compound, and by proxy the pulmonary platform, but we don't know if we will have enough data by key abstract deadlines to present data at scientific conferences this year.

I want to talk briefly about which CF patients we hope to help. Clearly there has been an enormous amount of progress over the last several years in CF treatment options. But there are still opportunities to:

      (a) help those who don't respond to standard of care; and

      (b) to make those that do respond even better.

The gene target of ARO-ENaC is the epithelial sodium channel, or ENaC. There is good genetic validation that CF patients who are also essentially heterozygous ENaC knockouts have a mild form of CF or even no discernible lung complications several decades into life. We see this as an indication that therapeutic ENaC inhibition in the lung may benefit all patients with CF,

regardless of the genotype. The idea is that reduction of ENaC expression in the lung helps to rehydrate CF-related dehydrated mucus and may help improve mucociliary clearance.

Importantly, this first study is designed to give us a readout on both tolerability and efficacy in the target patient population. This could be a potentially important value inflection point for both ARO-ENaC and for the pulmonary platform, broadly. If the data are supportive of further development we hope to launch a Phase 3 study in 2021 and move to rapidly expand the pipeline with product candidates that address other underserved pulmonary diseases, such as COPD, asthma, and pulmonary fibrosis.

Toward that goal, I'm pleased to announce that we have completed discovery and optimization work on our second lung-targeted program and have nominated ARO-Lung2 as our next candidate. We are not disclosing the target at this point, but we can say that it is designed to address COPD patients. We have been very encouraged by the data in this program and for ARO-ENaC and are eager to begin the expansion of our pulmonary franchise. We are now working on manufacturing and IND-enabling toxicology studies of ARO-Lung2 and plan on filing a CTA in the first half of 2021 to begin first-in-human studies. We had previously hoped this could happen by the end of the year, but COVID has slowed development down a bit and will delay first in human studies by probably 1 or 2 quarters.

In the past, we have discussed our excitement in establishing a rapidly-expanding pulmonary franchise, and you are seeing that start to play out now. Given our studies in rodent, primate, and sheep models spanning several years, we have a high degree of confidence that our inhaled delivery will be well-tolerated and active in humans. Further, the lung represents a target-rich environment that

enables us to address a number of indications in innovative ways, so we see this as a big opportunity for patients and a significant value driver for our shareholders.

In addition to CF, COPD, asthma, and IPF, people have asked us about applying inhaled TRiM™ to corona virus. We have not disclosed any work previously, but are happy to report today that we have an active program to address the current novel coronavirus that causes COVID-19 and other possible future pulmonary-borne pathogens. We are not disclosing more details about this program or strategy, but we wanted to provide this update. We are looking to bring the same ingenuity and innovative thinking to this issue that we did in revolutionizing the approach to Hepatitis B. All of us who work in biopharma and drug development play a role in improving global health and Arrowhead is proud to say that we have joined the fight. A number of factors give us confidence that we could play an important role in the current novel corona virus, future corona viruses, and other pulmonary-borne pathogens:

- First, we are the clear RNAi leaders in addressing the lung and have a clinic-ready inhaled program;
- Second, history suggests that we are faster than any RNAi company, and arguably any other biotech company, going from concept to clinic; and
- Third, we are the leading RNAi company in antivirals and are known as HBV thought leaders.

We look forward to keeping you up to date on our progress.

The final program I would like to mention is our muscle-targeted program. We have not yet disclosed the initial indication or gene target of our first clinical candidate, but consistent with our other programs:

- we view the indication as having substantial unmet medical need;

- the gene target is well-validated, and
- we expect to be the first RNAi company there.

As with the solid tumor and pulmonary franchises, we view our ability to address skeletal muscle as the sharp end of a spear. Once we achieve clinical proof of concept, we expect to rapidly expand our pipeline into new indications and gene targets addressable via muscle delivery. We remain on track to file a CTA by the very end of 2020.

I believe we have unmatched reach into diverse indications, unmatched speed to the clinic, and unmatched depth of pipeline for a company our size.

Our partnered programs continue to look good. Amgen stated on its recent quarterly conference call that it expects to begin a Phase 2 study with AMG 890 in the second half of this year. Janssen continues to conduct its first two Phase 2b studies with JNJ-3989 against chronic HBV, and we are actively working together on the 3 undisclosed additional targets.

COVID-19 has introduced a new set of challenges, but Arrowhead is adapting. We believe that we have important new medicines that can potentially help countless patients, so we feel very fortunate that the current environment has only caused minor delays to our development programs and that we are well resourced. Our employees continue to be a great inspiration to me as their focus, work ethic, and innovative spirit have never waned during these difficult times.

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

Javier San Martin

Thank you, Chris. I'm happy to join the call and hope I can be a good resource to everyone listening today.

Chris gave a good overview of the clinical development programs. I will provide some further details on their status and study designs.

Let's begin with ARO-APOC3, our candidate targeting apolipoprotein C-III, being developed as a potential treatment for patients with hypertriglyceridemia. As Chris mentioned, we believe this may be a good treatment option for MCM patients that have severely elevated triglycerides, often in the thousands of mg/dL. These patients can experience recurrent abdominal pain, are at high risk for pancreatitis, and in some cases can require frequent visits to the ER and be admitted for multi-day hospital stays. In the most severe cases of pancreatitis, these attacks can even be fatal. In addition, these patients live with a very restricted diet that becomes difficult to maintain, and even if they comply they still can have extremely high triglyceride levels. These patients have severely impacted quality of life and are desperately in need of better therapies that can achieve deep and durable reductions in triglyceride levels and therefore reduce the risk of pancreatitis and allow for a better quality of life.

As Chris mentioned, we previously announced some preliminary results in this patient population, but I want to review them today because they were very encouraging. After a single dose of 50mg of ARO-APOC3 in patients with severe hypertriglyceridemia, we demonstrated reductions of around 95% in circulating

triglycerides. We would expect this type of reduction to have substantial clinical benefits, particularly in patients with a history of pancreatitis.

We are currently conducting our Phase 1 single and multiple dose study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of ARO-APOC3. The single-dose portion of the study is in adult healthy volunteers and the multiple-dose portion includes patients with severe hypertriglyceridemia. 71 subjects have been enrolled and dosed in this study. We still have a few patients to go in order to reach the planned enrollment. We are working with investigators and sites to ensure that we reach this planned enrollment as soon as possible. We look forward to the opportunity to present additional results from this study later in the year.

Our other cardiometabolic candidate is ARO-ANG3, targeting angiopoietin like protein 3, or ANGPTL3, and is being developed as a potential treatment for patients with mixed dyslipidemia. This program is also moving forward efficiently.

ANGPTL3 is a regulator of lipid and lipoprotein metabolism. Inhibiting ANGPTL3 should result in lower triglycerides and LDL-C, and potentially provide improvements in other lipid and metabolic markers. Our data in animal models and our early clinical data strongly support that.

For example, at doses of 200 or 300 mg, maximum mean triglyceride reductions in the high triglyceride cohort approach 80% and maximum mean reductions in LDL cholesterol in the various high-LDL cohorts are averaging around 40%. These patients were already on maximal medical care consisting of statins plus or minus ezetimibe with PCSK9 inhibitors in some. Lowering both triglycerides and LDL to

this extent was an exciting result and we think could serve to hit multiple cardiovascular risk factors simultaneously.

The current clinical study is a Phase 1/2 single and multiple dose study to evaluate safety, tolerability, pharmacokinetic, and pharmacodynamic effects. The single-dose portion of the study is in adult healthy volunteers. The multiple-dose portion is in normal volunteers *and* in patients with various types of dyslipidemia. This includes patients with:

- hypertriglyceridemia;
- patients on a stable LDL treatment regimen but with persistently elevated LDL cholesterol;
- patients with heterozygous or homozygous familial hypercholesterolemia;
- and patients with non-alcoholic fatty liver disease.

We have enrolled and dosed 93 subjects in this study and have reached full planned enrollment.

The data for both ARO-APOC3 and ARO-ANG3 strongly support further development and we intend to find appropriate ways to share the data publicly this year.

Earlier, Chris discussed some of our future plans for ARO-ANG3. It seems likely that a Phase 2b study that assesses various dose levels and dosing intervals would be a smart addition to our data package before embarking on a Phase 3 Cardiovascular Outcomes Trial. This will give us more certainty on the magnitude of treatment effect in a larger data set, allow us to select the right dosing regimen,

and also build our safety database. We are developing our strategy and plan to engage with regulators this year to discuss the development and regulatory path.

ARO-HSD, our new investigational candidate targeting HSD17B13 for the potential treatment of alcohol and/or nonalcohol related liver disease, is another exciting program that has made progress recently. We see this as the most intriguing target for NASH at the moment. Population based genetic data have shown strong protection against NASH cirrhosis, and alcoholic hepatitis and cirrhosis in humans that possess loss-of-function mutations in the HSD17B13 enzyme. We are eager to see how that translates therapeutically in patients that receive ARO-HSD treatment.

Our current clinical study is a Phase 1 single and multiple dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of ARO-HSD in normal healthy volunteers as well as in patients with NASH or suspected NASH. Additional exploratory objectives include assessment of various measures of drug activity using liver biopsy.

The first cohort of volunteers received their dose, and tolerability data was collected. The DSMB reviewed those data and recommended continuing dose escalation. The study will resume enrollment shortly, once some of the COVID-19 related restrictions are eased in New Zealand. We expect to begin enrolling the first cohort of NASH patients in the multiple-dose portion of the study after the review of safety parameters from the second cohort of healthy volunteers. This parallel design strategy makes the time to patient activity data far shorter than a traditional sequential Phase 1 SAD to Phase 2 MAD design. It's another innovative way that Arrowhead operates.

Lastly, I want to give an update on ARO-AAT, our second-generation investigational RNAi therapeutic being developed as a treatment for the rare genetic liver disease associated with alpha-1 antitrypsin deficiency. There are two ongoing clinical studies: the potentially pivotal SEQUOIA study and the open-label 2002 study. We voluntarily put both on a 4-week pause for new screening and enrollment due to concerns around COVID-19. Many alpha-1 patients have compromised lung function and may be at increased risk of severe illness in the event of COVID-19 infection. Continuing to enroll new patients might have also jeopardized the integrity of study data as patients could have difficulty completing study visits and could miss doses or relevant study related procedures as a result of travel restrictions or concomitant illness.

We are now working with participating sites to restart screening and enrollment where and when it is prudent to do so.

We are still on schedule to collect 6-month biopsies for cohort 1 of the 2002 studies by the end of the summer. We will then work on processing and analyzing results, and subsequently plan on sharing those data in an appropriate venue. This may be the first liver biopsy data of patients treated with any therapy designed to address alpha-1 liver disease. We will be looking intently to learn what happens to Z-AAT monomer levels and accumulated Z-AAT polymer, in addition to other possible measures.

I will now turn the call over to Dr. Curt Bradshaw, Arrowhead's Chief Scientific Officer. Curt?

Curt Bradshaw

Thank you, Javier and good afternoon, everyone. I'm pleased to meet you all virtually and hope to connect more when things ease up a bit.

I want to give a little color on what we're working on in our early programs and how we're addressing resource needs in research and manufacturing.

First, from a discovery standpoint we have a lot of programs in active development and more in the planning stages. As Chris mentioned, ARO-Lung2 has already been nominated and we are now in the manufacturing and IND-enabling study phase for that program. We have been highly encouraged by our non-clinical results with ARO-ENaC and Lung2, so we are moving as quickly as possible into new targets that leverage our success with the pulmonary TRiM™ structure.

This includes some ideas and initial work on pulmonary infectious diseases, like the novel corona virus that causes COVD-19 and other corona and non-corona viruses where knockdown of a target in the pulmonary epithelium may be helpful. The idea of a long-duration intervention, which RNAi generally has demonstrated, gives some unique advantages over other approaches. Our inhaled delivery platform has been developed and optimized over the last several years, so we think we have a significant leg up over other potential RNAi solutions.

Moving on to our other more advanced preclinical efforts. In the liver, we are working towards several new programs. Importantly, these include new targets as well as possible dimer, or bispecific, programs designed to silence two gene targets with a single drug candidate.

In muscle, we have 1 lead program and another 2 that are in active development but earlier stage.

So, how are we building and allocating resources to support these plans? One of the positive aspects of being a company built around a single mechanism and a single scalable platform, is that learnings from one program tend to inform the development of others. This is absolutely true for the Arrowhead research team. We have not needed to drastically increase headcount and are only selectively adding when specialized expertise is needed. For example, we are adding a few folks to support the growing pulmonary area.

There will be some additional needs for some of the new cell types we are working on. This is partly why the new San Diego R&D facility, which we opened last month, is helpful. It allows us to tap into additional skill sets in one the country's premier biotech hubs. It has also expanded our capacity for preclinical models, so we are able to do more of the early work in parallel.

Lastly, I want to touch on our manufacturing capabilities and what we are seeing in this COVID-19 environment. We are monitoring the situation very closely because we never want drug manufacturing to delay a clinical study. So far we have not seen any supply disruptions. We have not encountered any material delay in receiving raw materials needed for the manufacturing process and have not seen any contract manufacturer delays either.

We utilize a combination of internal GMP manufacturing and external CMOs. We typically manufacture all material for pre-clinical tox studies and Phase 1 clinical studies in house and then use CMOs for Phase 2 and beyond. We maintain redundancies both internally and with various CMOs in different geographies in order to guard against the risk of supply disruptions. So, to summarize, we think

we are probably in a good position to supply the needs of our clinical development team now and into the future, even with the uncertainty of COVID-19.

I will now turn the call over to Ken Myszkowski, Arrowhead's Chief Financial Officer. Ken?

Ken Myszkowski

Thank you Curt.

As we reported today, our net loss for the quarter ended March 31, 2020 was $19.8 million or $0.20 per share based on 101.7 million fully-diluted weighted average shares outstanding. This compares with net income of $23.9 million, or $0.24 per share based on 98.1 million fully-diluted weighted average shares outstanding, for the quarter ended March 31, 2019.

Revenue for the quarter ended March 31, 2020 was $23.5 million, compared to $48.1 million for the quarter ended March 31, 2019. Revenue in both periods relates to the recognition of a portion of the upfront payments and milestones received from our license and collaboration agreements with Janssen, as we continue to work toward completing our performance obligation of managing the current phase 1/2 HBV clinical trial. Revenue from the Janssen agreement is recognized based on our estimate of the proportion of effort expended toward fulfilling our performance obligations – primarily, overseeing the completion of the current phase 1/2 HBV clinical trial. We expect the remaining $33.2 million of deferred revenue to be recognized in this calendar year. Any additional milestones achieved with Janssen or Amgen would be additive to this projection.

Total operating expenses for the quarter ended March 31, 2020 were $45.8 million, compared to $26.1 million for the quarter ended March 31, 2019. This increase is primarily due to increased non-cash stock compensation expense. Stock compensation expense has increased because the valuation of new stock option and restricted stock awards granted has increased with the growth in our stock price. Additionally stock compensation expense increased due to the timing of the achievement of certain performance-based awards in each period. The increase in total operating expenses was also driven by increased clinical trial costs as our pipeline of clinical candidates has increased, and increased personnel costs in both R&D and G&A as our headcount continues to grow.

Net cash used by operating activities during the quarter ended March 31, 2020 was $27.6 million, compared with net cash used by operating activities of $19.6 million during the quarter ended March 31, 2019. The increase in cash used by operating expenses during the quarter is consistent with the increase in our cash operating expenses. We estimate our near-term cash burn to average $30-35 million per quarter.

Turning to our balance sheet, our cash and investments totaled $498.2 million at March 31, 2020, compared to $302.9 million at September 30, 2019. The increase in our cash and investments was primarily due to the December 2019 equity financing we completed, which generated $250.5M in net cash proceeds for the Company.

Our common shares outstanding at March 31, 2020, were 101.7 million.

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

> **Chris Anzalone**

Thanks Ken.

COVID-19 has caused everybody to rethink how they operate in their personal and professional lives. We at Arrowhead are not immune to that. We are committed to protecting our employees, business partners, and patients that participate in our studies, and have taken decisive action towards that goal. From an investment standpoint, we are encouraged that the outbreak has not, so far, caused wide scale disruptions to our business and at worst we have seen some minor extensions to anticipated development timelines. Importantly, we are excited to be leveraging our leading inhalation franchise and actively working on treatments for the current novel corona virus as well as future pulmonary viruses. This is directly in our wheelhouse and we are confident that we have much to offer.

In addition, we believe 2020 holds great potential to be an important year for the company in terms of:

- expanding our reach into new indications; and
- continuing to validate the TRiM™ platform in hepatocytes, solid tumors, the lung, and skeletal muscle.

We intend to have readouts across most of our programs, and we expect these to be important data. We also expect to start to gain clinical proof of concept for our extra-hepatic platforms, begin the pipeline expansion phase for our growing pulmonary platform, expand the TRiM™ platform into new opportunities, engage with regulators to gain clarity on our plans to move our cardiometabolic candidates into pivotal studies, and maybe even have a breakthrough or two to discuss.

We have had equally ambitious plans in the past and we think we have a pretty good track record of meeting or exceeding those expectations.

Thanks again for joining us today. I would now like to open the call to your questions. Operator?

Operator

Operator opens the call to questions …