

ARROWHEAD PHARMACEUTICALS

Fiscal 2025 Second Quarter Conference Call – Prepared Remarks

May 12, 2025

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 2025 second quarter ended March 31, 2025.

With us today from management are president and CEO Dr. Chris Anzalone, who will provide an overview; Dr. Bruce Given, interim chief medical scientist, who will provide an update on our cardiometabolic pipeline; Andy Davis, senior vice president and head of global cardiometabolic franchise, who will provide an update on commercialization activities; Dr. James Hamilton, chief medical officer and head of R&D, who will discuss our earlier stage development programs; and Ken Myszkowski, our outgoing chief financial officer who is retiring this week, who will give a review of the financials. We also welcome Dan Apel, our incoming

CFO, who is also with us on the call today. Following management's prepared remarks, we will open the call to questions.

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.

Before I start, I want to say thank you to Ken and wish him the best in his retirement. Ken has been a valuable member of the Arrowhead team, and he retires at a time of great financial strength for the company. The finance organization that Ken built over the years is very capable and provides strong support to our ambitious development and commercialization plans. From all of us at Arrowhead, thank you for all the important contributions over the last 16 years.

I am also excited that Dan Apel will join us as our new CFO at a critical time for Arrowhead. We expect to make the transition from development stage to commercial stage, with the planned launch of plozasiran this year, pending regulatory review and approval. Dan is an accomplished pharmaceutical executive who can make an immediate and important impact on our business.

Let's now talk about our business and the progress we're making toward our short, mid, and long-term goals. Arrowhead is at an important point both in terms of capabilities and potential value as we drive our organization toward our first commercial launch, which we anticipate this year. Following this, we expect multiple additional independent and partner launches over the following few years. The combination of:

- commercial expansion,
- our extraordinarily productive discovery engine,
- the increasingly validated nature of our platforms and RNAi modality,
- our large pipeline of clinical-stage assets,
- our strong balance sheet,
- and clear access to additional non-dilutive capital

together provide us with a level of upside potential *and* stability that I believe is a rarity in our industry. This is always attractive, but is even more valuable at a time when biotech markets have been depressed for the past several years and near-term capital markets are uncertain at best. As the current biotech market weakness causes people to weigh the tradeoff of stability versus the potential for explosive value growth, I think we have the tools for both.

I view our value proposition in layers.

Layer 1 is Plozasiran. It constitutes our primary near- and mid-term value driver and provides a strong base for us. Plozasiran has shown to be a potent triglyceride-lowering agent across multiple clinical studies in hundreds of patients. We believe there are 3 – 4 million people in the US alone who suffer from severe hypertriglyceridemia (or SHTG), as defined by fasting triglyceride levels above 500 mg/deciliter. We are preparing to launch into a small subgroup of this population, patients with familial chylomicronemia syndrome (or FCS), and have a PDUFA date of November 18, 2025. We also completed the submission of a Marketing Authorization Application or MAA with the EMA and are working through additional planned submissions in other select geographies.

The P3 data supporting our regulatory submissions were consistent and encouraging. Genetically-defined and clinically-defined FCS patients responded similarly, with reductions in tryglycerides of about 80% from baseline; approximately 75% and 50% of patients had triglycerides go below 880 and 500 mg/dl, respectively, which are discussed in guidelines and the academic literature as important goals for minimizing pancreatitis risk. These are truly impressive levels to achieve in FCS patients, as the mean baseline triglyceride level in the study was approximately 2500 mg/dL. Plozasiran was generally well-tolerated and showed triglyceride reductions in 100% of patients treated at the primary endpoint of 10 months.

Our hope of treating FCS patients is important. This is an historically underserved population and we believe plozasiran could be an important medicine for them. However, we view this as just the beginning.

SHASTA-3, SHASTA-4, and MUIR-3 are P3 studies designed to support a supplemental NDA and other applications on a global basis to enable us to treat the

broader SHTG patient population. These studies are moving rapidly and we believe they could be fully-enrolled this summer. We are also in the process of initiating SHASTA-5, which is an outcomes study to specifically evaluate the risk reduction of acute pancreatitis in high-risk patients with SHTG. We think this is an innovative strategy to potentially demonstrate meaningful value for patients, physicians, and payors.

Our second layer of value may be our initial obesity candidates, and initial CNS candidates. Regarding the former, ARO-INHBE is currently dosing in obese patients and we expect ARO-ALK7 to begin dosing in obese patients shortly. Both are designed to intervene in a biological pathway regulating fat storage. ARO-INHBE targets hepatocytes with the same TRiM™ platform used in several ongoing clinical studies that has been in thousands of patients. It is designed to reduce hepatocyte expression of Activin E, which is a ligand for adipose ALK7. ARO-ALK7 is the first in industry adipocyte-targeted siRNA with a new TRiM™ platform that, in animal models, has shown good uptake in adipose tissue and high levels of target gene knockdown with a long duration of effect that may enable Q4 month, Q6 month, or less frequent administration. ARO-ALK7 is designed to reduce expression of the ALK7 receptor itself in adipose tissue.

Both programs demonstrated substantial reductions in visceral fat mass versus control while simultaneously preserving lean mass in animal models. Both targets are also supported by human genetics, where loss-of-function carriers have favorable body composition and metabolic characteristics compared to non-carriers, without any apparent safety cost. It's a very intriguing pathway that we believe may fill some important gaps left by standard of care obesity treatments, addressing some of the shortcomings of the GLP1/GIP class. The possibility of

long-acting agents that are well tolerated, spare muscle mass, and enable visceral fat loss *without* dependence on caloric-restriction is exciting.

ARO-INHBE began dosing a Phase 1/2 study in December 2024, and we anticipate having some initial data by the end of 2025. As I mentioned, we expect ARO-ALK7 to begin dosing shortly and we could have some initial data soon after ARO-INHBE results become available. Studies in both candidates include single dose and multiple dose monotherapy arms in obese subjects, as well as multiple dose arms that include combination with tirzepatide.

Our CNS BBB platform has made great strides in recent years. We have a substantial amount of preclinical data across multiple models that make us optimistic that we can deliver potent RNAi drugs to the brain via a simple subcutaneous injection. Delivering large molecule drugs systemically and getting past the blood brain barrier has been a holy grail virtually as long as complex biological drugs have been developed and we expect to be in the clinic late this year. Our first candidate, ARO-MAPT, targets the Tau protein for potential treatment of Alzheimer's. We expect to follow that with ARO-HTT, licensed to Sarepta, against Huntington's Disease by the end of the year. In the first half of 2026, we expect to bring ARO-SNCA to the clinic, which targets alpha-synuclein for potential treatment of Parkinson's. These are all well-validated targets against very important diseases for which effective agents have long been sought and we look forward to seeing how they translate from animal models to humans.

A third layer of value could come from our other phase 3 drugs. We expect to begin enrolling a year-long P3 study of zodasiran for homozygous familial hypercholesterolemia (or HoFH) shortly. The HoFH patients treated with zodasiran in P1 and P2 studies gives us confidence that we may have a potent

LDL-C-lowering agent that only requires quarterly dosing in this important at risk patient population. The sales infrastructure we are building for plogasiran could easily be leveraged for this population, so this feels like a straightforward, relatively rapid, low-risk, and low-cost expansion of our commercial presence.

Fazirsiran is our drug candidate against AAT liver disease. Our prior studies give us confidence that it could be an effective agent to reverse fibrosis in this largely unserved patient population. Fazirsiran is partnered with Takeda and they have publicly guided that the Phase 3 studies could complete enrollment this year. They are 2-year studies to primary endpoint. While this is partnered, our economics are substantial, with 50-50 profit share in the US, 20-25% royalties X-US, and up to \$527m of remaining milestones.

While we view these as our primary near- and mid-term value drivers, there are substantial pieces of our business underneath them providing redundancy and additional upside potential. They include:

- 4 wholly-owned additional P2-ready clinical programs in ARO-C3, ARO-CFB, ARO-RAGE, and ARO-PNPLA3
- 2 P2 programs partnered with GSK: against chronic hepatitis B infection and MASH
- Another P3 program partnered with Amgen, in olpasiran
- 4 P1/2 clinical programs partnered with Sarepta
- 3 designated preclinical programs partnered with Sarepta, one of which I already mentioned, in HTT
- and 6 additional preclinical programs to be named by Sarepta

And of course underlying all of this is a discovery engine that we believe is second to none in the siRNA field. We expect this to continue to drive value as the basis for many additional wholly-owned drugs and through future partnerships.

With all these layers, one can reasonably ask how many of these would be required to create a large, productive, sustainable pharmaceutical company. We indeed have many opportunities to create durable value.

Importantly, we believe we have the capital and access to substantial additional capital to support our work. The Sarepta deal was a critical component of this. During the last quarter, we closed the global license and collaboration agreement with Sarepta Therapeutics, materially strengthening our balance sheet. This transaction brought in \$500 million as an upfront payment and \$325 million through the purchase by Sarepta of Arrowhead common stock priced at \$27.25 per share. Arrowhead will also receive \$250 million to be paid in annual installments of \$50 million over 5 years. In the short term, we have the potential to receive an additional \$300 million in milestone payments associated with the continued enrollment of a Phase 1/2 study of ARO-DM1, which we are on track to achieve during the next few quarters. Taken together, this adds up to \$1.375bn in cash payments. The total potential value of this deal including upfront payments, equity investment, and potential milestones exceeds \$11 billion. We are also eligible to receive tiered royalties on commercial sales.

This would be a transformational deal in any environment. But, as I mentioned, with the state of biotech equity markets today, we feel very good about not having to raise equity capital at this time to fund our growth as we become a commercial company. We are now funded into 2028 and through multiple important milestones that we think can drive substantial value for our shareholders.

With that overview, I'd now like to turn the call over to Bruce Given. Bruce?

Bruce Given

Thanks Chris and Good Afternoon everyone.

Arrowhead has been working in RNA interference for nearly 20 years. During that time, we have made great strides creating a modality that is increasingly scalable, reliable, potent, and generally well-tolerated. We have also made great strides bringing RNAi to where it is needed. In addition to delivering to hepatocytes, we are now able to address lung, CNS, muscle, adipose, and cardiomyocytes. We have always been a great R&D platform company, and we are now taking a next step forward as we seek our first marketing approval for plozasiran in familial chylomicronemia syndrome or FCS.

Most of you will be aware of the results of our phase 3 Palisade study, which were published in the New England Journal of Medicine last year and showed statistically significant responses on all primary and alpha-controlled secondary endpoints, including a large reduction in the primary endpoint of triglyceride reductions at 10 months, as well as reduction in incidence of confirmed pancreatitis in the protocol-defined comparison of placebo and the combined 25 and 50 mg dose groups.

Following pre-NDA discussions with the FDA, a marketing application for approval for use in FCS was submitted on November 16, 2024 and accepted for review by FDA with a PDUFA date of November 18, 2025. At this time, we are not anticipating being asked to participate in an advisory committee meeting. We

have been asked frequently whether the changes in Washington have impacted the review. While we have no special insights into the inner workings of the Agency, our impression has been that the review is progressing as we would have anticipated and we know of no FDA personnel or timing changes affecting our program at this time.

We have also had routine pre-filing meetings with the EMA and appointed CHMP Rapporteurs that culminated in the submission of a Marketing Authorization Application or MAA on February 28, 2025, which was confirmed to be valid for review on March 20, 2025. Our plan is to seek approval in the UK following approval in either the US or Europe by leveraging the International Recognition Procedure (IRP). We have also had pre-filing meetings with the Canadian and Japanese regulatory authorities and have plans for filing marketing applications in those and other jurisdictions, as well. We are hopeful that these filings will lead to Arrowhead's first commercial launch, possibly beginning as early as late this year.

As welcome as we believe that plogasiran will be for FCS patients, this is just the beginning of the story for this important drug. While we were studying plogasiran in FCS, we were also evaluating the drug in much larger patient populations including severe hypertriglyceridemia or SHTG, defined as patients with fasting triglycerides above 500 mg/dL but without genetic FCS, as well as in patients with mixed hyperlipidemia. These important studies led to publications last year in the New England Journal of Medicine, JAMA cardiology and Circulation journals. After receiving End-of-Phase 2 feedback from FDA and EMA, we have initiated a Phase 3 program in severe hypertriglyceridemia. This program is designed to meet key standards based on guidance documents from the International Council for Harmonisation or ICH. Key requirements that drove design considerations include

2 pivotal placebo-controlled trials in SHTG patients and a safety database of at least 1500 patients treated with plogasiran compared with placebo for 12 months.

Hence SHASTA 3 and 4 are very similar studies designed to demonstrate statistically significant improvement in triglycerides with 25 mg plogasiran compared with placebo over 12 months of treatment. The 2 trials total around 700 patients and are very highly powered, given the results seen in the Phase 2 SHASTA-2 trial where the primary endpoint of difference in triglycerides at week 24 compared to baseline at the 25 mg dose was -53 % with a p value of <0.0001. To reach the necessary 1500 patients for the safety database, SHASTAS 3 and 4 are supplemented by a supportive Muir-3 trial in mixed hyperlipidemia, also blinded and comparing 25 mg quarterly plogasiran versus placebo for 1 year with a planned enrollment of ~1400 patients. We have previously guided that we expect the last patient to be randomized in the SHTG program this year. Based on enrollment to date, we now anticipate the last patient to enroll sometime this summer. We have been encouraged by the enthusiasm of our investigators, as indicated by the rapid enrollment, and also by a very low premature discontinuation rate for adverse events and other reasons. The last patients entered will be treated for 1 year before the database can be locked, the data analyzed and hopefully submissions made to regulatory authorities seeking approval for use in SHTG patients. Thus, before the end of this summer, it should be possible to narrow the potential timing for SHTG supplemental NDA submission.

The SHTG program also features a first of its kind study named SHASTA-5 to directly assess the ability of plogasiran to reduce the risk of acute pancreatitis in SHTG patients with a purpose-designed outcomes study. We are conducting this study expressly to meet the needs of sophisticated payors, especially outside of the US. It is not gating for the SHTG filings and is unlikely to be complete prior to

those submissions. We do hope that it will be completed during the review process in Europe and elsewhere to be available for pricing discussions with national payors post-approval. We cannot provide precise timing expectations not only because of uncertain timing to complete enrollment but also because as an outcome study, treatment will continue until the required number of events have been collected, rather than for a fixed duration. Screening is underway at centers currently open for enrollment, so the trial is underway. More information on design will be presented following presentation at a major medical meeting.

The R&D group is also playing its role in growing the awareness that new treatments are reaching the market for treating FCS. Our Medical Affairs team reporting into R&D, continues to play a vital role in educating the medical community. Our Medical Science Liaisons are actively engaging healthcare professionals in scientific exchange, helping them better understand and raising awareness of familial chylomicronemia syndrome, the significant unmet medical need, and the growing body of clinical data now available.

The team has been present at key medical congresses in the quarter, including the American College of Cardiology meeting, the European Atherosclerosis Society and British Atherosclerosis Society joint meeting, and the European Society of Endocrinology meeting taking place presently. In parallel, our Publications team continues to generate and disseminate important data to support these scientific efforts and expand awareness within the clinical community. Most recently, at EAS, an abstract authored by experts in the field titled: “PALISADE: Plozasiran Decreases Risk of Acute Pancreatitis and Improves Indices of Quality of Life in FCS” was featured. The authors concluded in the abstract that in patients with FCS, Plozasiran markedly reduced TGs and risk of acute pancreatitis with promising changes in indices of quality of life.

Those of you who have watched us for a while know that we have another agent in our cardiometabolic pipeline that, like plozasiran, has very strong support from human genetics. I'm describing zodasiran, an RNAi drug designed to reduce expression of Angiopoietin protein like 3 or AngPTL3. This drug also produced strong results in two Phase 2s including ARCHES-2, also published in NEJM last year as well as the GATEWAY study in HoFH patients, with data presented last week at the European Atherosclerosis Society conference. However, as we've discussed previously, we saw zodasiran as positioning in the crowded LDL space where the unmet need was largely being addressed by statins and PCSK9 inhibitors. But there is an important population where we think zodasiran would make an important contribution without requiring an outsized commitment of resources by Arrowhead, this being for patients with homozygous familial hypercholesterolemia. These patients have exceptionally high LDL cholesterol levels and because their genetic abnormalities usually result in very low or absent LDL receptor function, are usually not able to get to goal LDL levels even with maximum statin and PCSK9 therapy. This leaves them with very high risk of suffering cardiovascular events and early mortality.

A monoclonal antibody against AngPTL3 has already been proven effective in these patients but requires monthly intravenous injections and can lead to immunologic reactions. We conducted an exploratory phase 2 study in this population and saw similar benefits but with convenient quarterly, subcutaneous dosing. Moreover, these patients are usually cared for by physicians which largely overlap the physicians that treat FCS and SHTG, making the potential marketing of zodasiran in these patients efficient for us, were it to be approved. We have designed a phase 3 study of similar size to our phase 3 palisade study comparing

zodasiran 200 mg quarterly to placebo and expect to be enrolling patients this year. Assuming successful demonstration of safety and efficacy and successful regulatory submissions, zodasiran could join plozasiran in the market as early as 2028 or 2029.

As I said at the beginning of my remarks, we hope to see plozasiran emerge as our first commercially available drug to treat FCS patients as early as this year. Based on our expected completion of enrollment in the SHTG phase 3 program this summer, we could see SHTG Phase 3 completion in the summer of 2026 and an sNDA filing shortly thereafter. Interestingly, also before completion of the decade based on public guidance, progress with Phase 3 partnered programs for olpasiran in Lp(a) and fazirsiran in the liver disease associated with alpha-1 antitrypsin disease, could result in approvals for these programs in a similar time frame, as well, with the possibility that some of our less mature partnered programs for important orphan diseases might also find approval in that timeframe.

It takes patience in this business to see an important new platform rise to prominence. We feel there is some good reason to have confidence that RNA interference will join small molecule drugs and monoclonal antibodies as a foundational technology in drug development, especially as the field, which we see Arrowhead still leading, continues to push into new cell types, opening up additional important diseases to be addressed.

I will now turn the call over to Andy Davis.

Andy Davis

Thank you, Bruce.

With the PDUFA date for Plozasiran set for November 18th, just six months away, I'm pleased to share that our commercialization efforts are advancing rapidly and with strong momentum.

As discussed by Bruce, the Medical Affairs team is in the field helping educate the physician universe and facilitating publication of the results of Arrowhead's clinical trials.

We are making strong progress in building our commercial sales team. Our National Sales Leader and a full complement of Regional Sales Leaders are now on board and focused on hiring and on-boarding top-tier talent with deep rare disease and therapeutic area expertise. Interest has been very encouraging — with thousands of resumes in the queue — and we are on track to fully hire and train our sales force by late summer, ensuring ample time for target validation and disease state education in advance of launch.

Our Market Access team is executing effectively on our Pre-Approval Information Exchange, or PIE, strategy directed toward health care decision makers to help them plan for our potential approval. We've now engaged with payers representing a significant number of U.S. covered lives, delivering compelling content on the clinical value and anticipated profile of Plozasiran. We are encouraged by their interest in Plozasiran, especially regarding its potential to reduce triglycerides and acute pancreatitis risk.

Additionally, our Analytics team is deploying innovative technologies to identify individuals potentially living with FCS. We are connecting with these patients

through disease state education efforts, including opportunities for them to opt in for continued communication and support.

Across our research and stakeholder engagement, the clinical attributes of Plozasiran continue to resonate strongly. Our market research suggests that Plozasiran's deep and durable triglyceride reduction is compelling to numerous stakeholders. The PALISADE study showed that Plozasiran reduced triglycerides by ~80% from baseline as early as month one, with this effect sustained over 12 months and with limited variability while placebo subjects showed variable changes ranging from +10 to – 18%. As a reminder, the primary end point in PALISADE was the median percent change from baseline in fasting triglyceride levels at month 10, where Plozasiran demonstrated a placebo adjusted change of -59% with the planned commercial 25 mg dose.

Our market research also suggests that healthcare providers, caregivers, and patients have a strong desire to see triglyceride levels fall below expert guideline thresholds, such as 880 mg/dL and even 500 mg/dL. In PALISADE, approximately 75% of patients at the 25 mg dose achieved levels below 880 mg/dL, and approximately 50% achieved levels below 500 mg/dL. Numerous expert guidelines emphasize the importance of maintaining triglyceride levels below 500 mg/dL as the aspirational goal to reduce acute pancreatitis risk.

Finally, we've received feedback that patients are looking for a treatment option that minimizes disruption to their lives. Plozasiran shows a favorable dosing and safety profile. As a reminder, Plozasiran is conveniently administered once every three months, potentially minimizing treatment burden and improving adherence.

As our U.S. launch preparations continue at full speed, we are equally pleased to report steady progress on our European commercial readiness efforts. We have already established a field medical presence and are actively engaging in scientific exchange at key European scientific meetings — laying a strong foundation for a successful rollout. We remain on track and deeply motivated by the opportunity to bring investigational Plozasiran to individuals living with FCS and their families in both the United States and priority countries outside the United States. We believe this potential first-in-class siRNA therapy will mark a major advancement, and we are fully committed to unlocking its patient impact.

I will now turn the call over to James Hamilton.

James Hamilton

Thank you, Andy.

First, I'd like to provide a status update for our two early-stage obesity programs, ARO-INHBE and ARO-ALK7. ARO-INHBE is designed to reduce expression of Activin E, which is a ligand for adipose ALK7, while ARO-ALK7 is designed to reduce expression of the ALK7 receptor itself, both of which are involved in regulating adipose storage of fats. These programs both have the potential to reduce visceral fat mass while simultaneously preserving lean mass, which we demonstrated in preclinical models and are now evaluating in clinical studies.

ARO-INHBE began dosing in December of 2024 and is progressing on our planned timeline. As a reminder, the Phase 1/2 study will evaluate ARO-INHBE monotherapy administered to obese, otherwise healthy volunteers in both single and multiple dose escalation cohorts. The SAD cohort dosing is now complete and

the multi-dose monotherapy cohorts are actively enrolling. The study is also evaluating multiple doses of ARO-INHBE in combination with tirzepatide and these combination cohorts are actively enrolling now on our planned timeline.

We anticipate the ARO-ALK7 clinical program will be up and running shortly. The design of this study is very similar to the ARO-INHBE study with SAD and MAD monotherapy cohorts and MAD cohorts in combination with tirzepatide. This study will also enroll obese otherwise healthy volunteers.

Both studies are designed to assess safety, tolerability, pharmacokinetics, pharmacodynamics, and multiple exploratory obesity efficacy endpoints. We anticipate that some initial data may be available for ARO-INHBE around the end of 2025 and potentially for ARO-ALK7 shortly thereafter.

Our muscle targeted programs partnered with Sarepta, ARO-DUX4 for FSHD and ARO-DM1 for myotonic dystrophy type 1, also continue to make good progress in the Phase 1/2 studies, which are ongoing. While the decision to release data will be made jointly with Sarepta, it is our expectation that initial data release is possible in 2025.

Lastly, I want to highlight some topline results from Part 2 of a Phase 1/2 clinical study of ARO-C3, designed to reduce liver production of complement component 3 as a potential therapy for various complement mediated diseases.

In patients with IgA nephropathy, or IgAN, ARO-C3 achieved deep and sustained reductions in alternative pathway complement activity and proteinuria. The maximum mean reductions in C3 was 89% with serum AH50, which is an alternative pathway hemolytic assay, reduced by 85%. ARO-C3 also led to an

important improvement in proteinuria, with a mean reduction in spot UPCR of 41% and maximum individual reduction of 89% from baseline by week 24.

ARO-C3 was generally well-tolerated and the observed duration of effect is supportive of once every three months or less frequent subcutaneous dosing. We are very pleased with these results and are planning to present a fuller dataset at the upcoming European Renal Association, or ERA, Congress in June.

I will now turn the call over to Ken Myszkowski.

Ken Myszkowski

Thank you, James, and good afternoon everyone.

As we reported today, our net income for the quarter ended March 31, 2025 was \$370.4 million or \$2.75 per share based on 134.5 million fully-diluted weighted average shares outstanding. This compares with net loss of \$125.3 million or \$1.02 per share based on 123.3 million fully-diluted weighted average shares outstanding for the quarter ended March 31, 2024.

Revenue for the quarter ended March 31, 2025 was \$542.7 million. No revenue was recorded in the quarter ended March 31, 2024. Revenue in the current period relates to our license and collaboration agreement with Sarepta.

As you know, the agreement with Sarepta was significant, it included many assets, including clinical assets, preclinical assets and other assets to be developed. Accounting guidance requires that we allocate consideration to several items including the value of the licenses we transferred, the ongoing work of certain

clinical trials that we will oversee, as well as expected obligations regarding future assets to be developed. Initial fixed contract revenue to be allocated included the upfront payment of \$500 million, the premium paid on the stock purchase of \$84 million and \$250 million relating to the five-year annual milestone payments. This totals to about \$834 million, the majority of which was allocated to the license agreements and recognized immediately, and the balance will be recorded as we fulfill our performance obligations. We recognized revenue of \$542.7 million during the quarter ended March 31, 2025, and we expect the balance to be recognized over the period that we satisfy our performance obligations. These obligations include overseeing certain clinical trials, as well as performing R&D work related to future clinical candidates. We expect \$90 to \$125 million of revenue to be recognized over the next 12 months, solely related to revenue recognition of the initial fixed contract revenue. The balance will be recognized over the next 5 or so years, most of which will be recognized in the first half of that time period.

We also expect future revenue related to cost reimbursement for certain discovery and manufacturing activities.

Future near term milestones of \$300 million, related to the DM1 program, are expected to be earned in the latter half of the calendar year, and will be recorded in their entirety as revenue at that point, as would any other future milestone payments and royalties.

Total operating expenses for the quarter ended March 31, 2025, were \$161.5 million, compared to \$126.2 million for the quarter ended March 31, 2024. The key drivers of this change were increased candidate costs as the Company's pipeline of clinical candidates has both increased and advanced into later stages of development.

Net cash provided by operating activities during the quarter ended March 31, 2025, was \$460.1 million, compared with net cash used in operating activities of \$92.4 million for the quarter ended March 31, 2024. The increase in cash provided by operating activities is driven by the cash received for the Sarepta agreement.

Turning to our balance sheet, our cash and investments totaled \$1.1 billion at March 31, 2025.

Our common shares outstanding at March 31, 2025, were 138.1 million.

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

Chris Anzalone

Thanks Ken.

Arrowhead is in a strong and stable position as a business, and we have made meaningful progress towards our long-term goal of developing and ultimately commercializing new innovative medicines for millions of patients.

We are on schedule to launch plozasiran this year, pending regulatory approval, with what we think is a best-in-class profile with meaningful differentiation from currently available therapies in FCS. We are also well on our way to fully enrolling this summer our suite of Phase 3 studies designed to support regulatory submissions for the large SHTG patient population.

We are funded into 2028 and potentially through multiple launches of wholly-owned and partnered programs in late-stage development. In addition, we believe

our technology platform is the broadest and best in the field, giving us many opportunities to receive additional capital inflows from business development in areas that are outside of our core commercial focus.

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

Operator
