ArQule, Inc. (Basilea Transaction)

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Corporate Speakers:

- Peter S. Lawrence; ArQule, Inc.; President, COO
- Paolo Pucci; ArQule, Inc.; CEO
- Brian Schwartz; ArQule, Inc.; Chief Medical Officer

Participants:

- Jonathan Chang; Leerink Partners LLC; Analyst
- Chad Messer; Needham & Company, LLC; Analyst
- Matthew Cross; Jones Trading Institutional Services, LLC; Analyst
- George Zavoico; B. Riley FBR, Inc.; Analyst

PRESENTATION

Peter Lawrence: Good morning, everyone. Welcome to the ArQule investor conference call reviewing our clinical development strategy, including highlights from AACR and the transaction announced recently with Basilea Pharmaceutica Ltd. This is Peter Lawrence, President and Chief Operating Officer of ArQule.

Yesterday, we issued a press release that reported the licensing of worldwide rights, excluding the Greater China region, of our FGFR inhibitor, derazantinib, to Basilea. This release is available on our website at www.arqule.com.

Leading the call today will be Paolo Pucci, Chief Executive Officer of ArQule. Also present for the Company are Dr. Brian Schwartz, Head of R&D; and Rob Weiskopf, our Chief Financial Officer.

Before we begin, please note that we will be making forward-looking statements as defined in the Private Securities Litigation Act of 1995. These statements will include, among other things, projections regarding future milestones and royalty payments under the license agreement with Basilea and projections regarding the timing of key events related to the development of our ArQule's proprietary pipeline.

Actual results may differ materially from those projected in the forward-looking statements due to numerous risks and uncertainties that exist in ArQule's operations, development efforts and the business environment, including those factors discussed in our reports on Forms 10-Q and 10-K and other documents filed with the Securities and Exchange Commission.

The forward-looking statements contained in this call represent the judgment of ArQule as of today. ArQule disclaims any intent or obligation to update any forward-looking statements, except to the extent required by law. We will provide an opportunity for questions and answers at the end of this call.

I'd now like to introduce our CEO, Paolo Pucci.

Paolo Pucci: Thank you, Peter, and thank you all for joining us this morning. We would like to provide some context relative to the recently announced transaction with Basilea, and as well a number of clinical milestones that we have reported on during the AACR congress that is just winding out today in Chicago.

So I will -- Brian will begin today's call. We're providing specifically the update relative to the data we have presented at the AACR. I will then outline the general strategic context where the Basilea transaction should be framed. And then Peter will offer some details relative to the Basilea transaction.

So before further ado, I will pass on the call to Brian Schwartz for an overview of what we presented at AACR.

Brian Schwartz: Thank you, Paolo. As we illustrated during our Q4 2017 call, we have made significant progress with all of our assets in our pipeline. For derazantinib, we initiated a registration trial in intrahepatic cholangiocarcinoma in Q4 of 2017 with the objective of a potential foster market entry.

For miransertib, our lead AKT inhibitor, we are in advanced stages of planning for a registration program in proteus syndrome, also in collaboration with the NIH, the objective of achieving fast-to-market entry in this rare disease. In addition for miransertib, we also continue to recruit the Phase I/II trial in Overgrowth Diseases with the objective of expanding the rare disease strategy.

Lastly, for ARQ 531, our BTK inhibitor, we launched a Phase Ia/b trial in Q3 of 2017 and continue to recruit nicely patients with B-cell malignancies with the objective of achieving early proof-of-concept.

In addition, since our Q4 call, we have had further developments in our AKT franchise in oncology with data presented at AACR meeting in Chicago earlier this week. In particular, on Saturday, April 15, our scientific collaborators at Memorial Sloan Kettering Cancer Center had an oral presentation for a Phase 1b study, miransertib, in combination with the hormonal therapy, anastrozole, including a cohort of endometrial patients with PIK3CA or AKT mutations.

The data showed early signals of clinical activity with one complete response, three partial responses and two patients with stable disease from a cohort of eight patients with prior endocrine or platinum-based chemotherapy. The 150-milligram dose, five days on, nine days off, showed no serious adverse effects with no discontinuations or dose reductions.

For 751, our next-generation AKT inhibitor, we also presented, together with our scientific collaborators at MD Anderson Cancer Center, results from our Phase I dose

escalation study of ARQ 751 in adults with advanced solid tumors harboring AKT1, two and three genetic alterations activating PI3K mutations or PTEN. The data demonstrated a dose-dependent activity through the five cohorts presented to date, with promising signs of activity at the higher doses.

No dose-limiting toxicity or grade three or adverse events were seen at the highest doses tested. The data presented for miransertib in endometrial cancer leads us to further evaluate the potential of this drug in that setting with the recommended Phase II dose.

In parallel, we will also be able to begin the assessment of future plans with 751, including defining the therapeutic window as we approach conclusion of the Phase Ia trial. In summary, our AKT franchise is developing rapidly, both in oncology and rare diseases.

I will now turn the call over to Paolo to explain the strategic context and rationale for the derazantinib transaction.

Paolo Pucci: Thank you, Brian. So as it is apparent to all, it's all necessary to continually reassess the data flow, and put in that context the prioritization of investments we make. And in fact -- and this has become a more complex exercise since we have entered the rare disease franchise with our AKT inhibitor, miransertib, which is the leading of the two.

So we continuously evaluate which of our proprietary assets -- and so far, until the Basilea transaction was announced, everything was proprietary. We can and should develop independently to achieve meaningful inflection points. And then we also assess, at the same time, which asset will be best benefit, and at what point in time, from a strong partner.

And as part of this ongoing process that we run at ArQule, we concluded that more value could be created with derazantinib through our partnership with Basilea. In fact, we believe that Basilea has the resources not only to continue the registrational trial that we recently launched in intrahepatic cholangiocarcinoma, but it also has the resources to extend the clinical development program in iCCA and beyond -- something that we couldn't have done on our own, given the additional investment opportunities that we -- that have manifested to us through the latest data releases.

By partnering derazantinib, therefore, we believe that we have given this drug, which it's one -- is best-in-class FGFR inhibitor in our opinion, we've given this drug the greatest chance for success. And also, this puts ArQule in the position to concentrate incremental, financial and, importantly, operational resources. This is still a company with only 30-some people of staff at this point in time. We'll concentrate those financial and operational resources on the programs where we could create with our own resources significant value short to midterm.

To provide you with some general context on which we will elaborate further during our next quarterly call, we have -- with the data presented at AACR for the AKT franchise, we will make it a priority to add the resources to that franchise. Specifically, we will assess how to conclude in the most informative way the Phase Ia for 751 to answer the critical strategic question that Brian has posed. And then we will also strengthen our effort in expanding the rare disease program for ARQ 092. The project for completing the endometrial data set in -- with ARQ 092, it's already ongoing, and has been for a while.

We also believe that it's time that we start to prepare and invest in the preparation of the Phase 1b, of the Phase Ia trial that we have -- our a/b trial that we have ongoing for ARQ 531. As Brian mentioned, the study -- the first portion of that trial, the Phase Ia for the BTK inhibitor, ARQ 531, is proceeding nicely. And so we believe that this is the right time to begin to define the shape of the follow-up phase, which is the Phase 1b.

Both our AKT and BTK programs present very compelling development opportunities just like FGFR also in biomarker-defined patient population. So that remains a general theme for our clinical effort, and we believe that the data that has been presented at AACR further solidifies those prospects. The transaction we have announced therefore should be framed in that context. Advancing the opportunities that we have in our remaining proprietary asset and, giving derazantinib at the right time in its life, the opportunity to proceed with a more expansive program, albeit with a partner.

So to go over some of the transaction details, I would like to turn the call to Peter, and then we open up for Q&A. Please, Peter, go ahead.

Peter Lawrence: Okay. Thanks, Paolo. Turning to the transaction, we licensed to Basilea exclusive rights for derazantinib in all parts of the world, other than the People's Republic of China, Hong Kong, Macau and Taiwan, where we have licensed already the rights to Sinovant Sciences Ltd., a subsidiary of Roivant Sciences Ltd.

In connection with the granting of the license, Basilea will make an upfront payment to ArQule of $10 million. ArQule is also eligible to receive a milestone payment of $3 million if the ongoing registrational trial of derazantinib and second-line iCCA meets certain predetermined milestones prior to its conclusion.

In addition, we're also eligible to receive up to $323 million in regulatory and sales milestones and tiered royalties on net sales ranging from single digits to mid-teens.

In addition, under certain conditions, ArQule could have the opportunity to commercialize derazantinib directly in the United States. Basilea will be responsible for research, development, manufacturing and commercialization and all costs associated therewith, and will reimburse the Company for out-of-pocket costs and the cost of FTEs in connection with our systems related to manufacturing and development activities during the transition period.

From an operational standpoint, this transaction will have a positive impact not only on our cash position, but also on expenses going forward. As a result, we'd like to offer updated financial guidance for year-end cash for 2018. ArQule previously expected to end 2018 with between $23 million and $25 million in cash and marketable securities, and now expects to end 2018 with between $40 million and $42 million in cash and marketable securities, which would fund the operational plan into 2020.

Further details will be available at the time of our Q1 2018 earnings call, which is coming up on Monday, May 7th.

With that, we'd like to open up the call for questions. So, Operator, please open the call for Q&A.

QUESTIONS AND ANSWERS

Operator: (Operator Instructions)

Jonathan Chang; Leerink Partners.

Jonathan Chang: Can you talk about how you see the FGFR competitive landscape broadly? And what are the other FGFR tumor types that could be pursued with derazantinib beyond intrahepatic cholangiocarcinoma?

Paolo Pucci: The FGFR landscape is certainly a competitive one, but it's also one where derazantinib is vying for being right there, first-to-market with -- in the cholangiocarcinoma space certainly with the FGFR program of insight that has a similar study ongoing.

There is another FGFR inhibitor, if I recall correctly, with Johnson & Johnson, and that is in advanced stages for bladder, as well. The FGFR is implicated in a number of tumor types, and I believe that the strategy that's most widely deployed by the companies competing in this field is that of testing across the board in this tumor type with basket trials. And that was one of the things that we were constrained to do by our limited resources.

We had applied for a basket trial, and we had some [single] generation ongoing, but not to the extent that was necessary to compete, I would say, vigorously. So I think to answer the second part of your question, the opportunity for expanding the program is vertically by looking at early lines of therapy in cholangiocarcinoma, obviously, because you would access patients that could offer a longer duration of therapy. One could think about some form of adjuvant settings.

Obviously, Johnson & Johnson has responded in the way very strongly to the bladder cancer space. Urothelial and gastric are other opportunities that we have considered in the recent past, and that the scientific community considers amenable to FGFR treatment.

Now one comment I have made is about the antibodies to FGFR. We don't follow that field that closely to make a particularly informative comment on the FGFR antibodies.

Jonathan Chang: Can you also talk about how you're thinking about potential additional business development deals for the rest of your pipeline? What is the strategy there?

Paolo Pucci: The strategy evolves all the time on the basis of the opportunities. We view some assets as both strategic and assets where we can generate significant value with the resources we have.

I'll give you one as, first, the rare disease program for our AKT inhibitor, miransertib. The number of phases needed to deploy a registrational program for proteus syndrome is a relatively small number. The complexity of such trials is not to be underestimated, but its few patients we have been working in the field for a long time now for two years plus. We have the opportunity to leverage the partnership we have established with the NIH. So we have the resources, we have the competencies to bring that forward, and we believe the same is for the expansion strategy.

In fact, let me say that the Phase II company-sponsored trial that Brian has been conducting in the US and Italy in the broader family of Overgrowth Diseases, part of our strategy to expand down the road, the scope of our rare disease program is recruiting very nicely. We just recently opened up a second site in Italy, and they had already prescreened a number of patients that are coming on the trials as we speak.

So we would like as much as possible to maintain that program for ourselves. And the rationale for that is we have built expertise and we have the resources to implement, and the amount of resources to be dedicated to it is not particularly significant.

At the same time, I would say that the same reasoning applies to 531, so the BTK inhibitor for the foreseeable future. We are there as well just as with the rare disease program for miransertib, we are blessed with the partnership with the NIH. Here we have had a very strong partnership with one of the leading centers for BTK inhibition, which is Ohio State.

So the resources that we need, for an expansive Phase 1b trial -- we're just in the midst of the Phase Ia trial -- are now available in good part through this portfolio prioritization that we have done and through the partnering of derazantinib with Basilea. So 531 is also, for the foreseeable future, an asset that we have the ability to bring forward, and we have the ability and the skills and the capital to bring forward on our own.

Now ARQ 751 is a different question in oncology. Because if ARQ 751 really will demonstrate in the last stretch of the Phase 1a trial to be truly differentiated from the class, to be really next-generation AKT inhibitor with a superior therapeutic window compared to the AKT inhibitors currently in later stages of development, then the indications that are open for that kind of asset are very large indications. To give you a sense, the leading AKT inhibitor right now in development in oncology is in a

combination trial with anastrozole in prostate cancer for 1,000 patients. And it is also developing in breast cancer, too. Sorry, the combinations we have with (inaudible) are in prostate cancer.

So those are very large trials, very large because they are pointing to very large indications. Should we find ourselves in the position to have to look at those kind of opportunities, then we will see how to best address it, and then we will be open to consider potentially partnerships. But at this point in time, nothing is decided.

At this point in time, our focus is to implement the plan, as we have it, to support our new partner, Basilea, in continuing without interruption the iCCA trial. And then as the data mature for all the portfolio, we'll look at things again. But for rare disease and for BTK, we feel good about what we could do for the foreseeable future on our own.

Jonathan Chang: Can you help set investor expectations for the ARQ 531 updates expected later this year? And any color on how enrollment is going in that Phase I study?

Paolo Pucci: Well, enrollment, I would repeat what Brian has said, the enrollment is going nicely. And for that, we need to thank, of course, our internal staff, but also our clinical partners, beginning with the Ohio State University, which we visited just a week ago for such an update and other matters as well.

The expectations haven't changed since we discussed it during our last quarterly call. We hope to be able to present at EHA maybe three cohorts worth of data. We just presented at AACR our very first cohort, and we gave an indication through that cohort -- that data -- of what the initial PK and PD looks like for this drug, and it is, so far, in line with expectations. And then we hope to present, as much as we can of the trial also of 5, 6 cohorts, we hope, by ASH at the end of the year.

And obviously, we need to be left with some flexibility and decide what to present where because, sometimes, you can present prominently; sometimes, you can present less prominently. So we have to see what kind of slotting we get. For example, at EHA, is it going to be an e-poster? Or is it going to be proper poster? Is it going to be a discussion? So setting that aside that this is what we plan to do.

Importantly, I would say that we still hope that if the review process is successful, we will be able, sometimes around mid-year, to put in the public domain a vast body of data that we have accumulated over the years for 531. And that is a paper that will detail the per clinical experiment conducted for two years-plus by us and Ohio State in the CLL, as well as chemical structure and crystal structure of the drug. But unfortunately, I'm not in the mind of the reviewers of any of our papers that are out there now, and I cannot provide you with much better guidance than that.

Operator: Chad Messer, Needham.

Chad Messer: So we've talked about a lot of the pipeline here. I'm specifically interested in what the next steps for 751 are. You've talked about how potentially broadly applicable the drug could be, and that you want to get it into 1b. What does that study look like? What are the next things you want to look at? And how quickly do you want to get that into combinations?

Paolo Pucci: The answer to the last question is as quick as we can. We need -- the 751 trial, the Phase I trial has taken some time to get to this point for essentially three reasons. There's a lot of new people on this call today, so for their benefit, I hope the others will indulge. So we started a couple of years ago the Phase I trial for ARQ 751. We started with a relatively low dose. So that's taken some time to work the drug up to a relevant dose. We decided, for the financial constraints we had, to go with one site, but it's an excellent site because it is MD Anderson.

And then, we decided based on the body of knowledge we had at the time, and it was available in the scientific community for AKT utility, to go with already a biomarker-defined population at the onset in Phase Ia. And obviously, once you have to select biomarker in a Phase I trial, it's much more complicated. So these are the three reasons why the drug has taken a little bit more time than we had hoped to get to a dose.

Now as far as what we intend to do is, definitely, we know that AKT inhibitors are shining in combination. I just discussed recently that the thesis that underpins the leading AKT inhibitor now in oncology, which is essentially an AKT inhibition to standard of care in hormone-sensitive tumors, being those tumors [prostate], being those tumor breast, being those tumor endometrial -- we are participating in that path as well, it points to combination. However, we also want to explore completely the opportunity that this drug might also be a better single agent than those AKT inhibitors that have preceded them.

Brian, do you want to add anything about the 751 plans? We have maybe one more cohort to go?

Brian Schwartz: I think we are, based on our preclinical modeling and the early toxicity profile -- even though it's really grade one and a little bit of grade two -- we're starting to see toxicities at the doses that we're giving in the Phase I trial. So I think we're pretty close to a recommended Phase II dose with 751.

I just want to add one thing to what Paolo said, Chad, is as the biology is better understood in terms of how AKT inhibitors work against different mutations, we would really like to exploit that new knowledge in the Phase 1b expansion and quickly see if there is single agent activity that would warrant it moving forward as a single agent. If we do not see that activity level, we would move quickly into the combination setting like many other people have done.

Paolo Pucci: Many, but I would say not that many, because to our last count, there were roundabout six, seven AKT inhibitors in some form of post initial Phase I development. So AKT inhibitors are a relatively scarce commodity at this point in time.

Operator: Matthew Cross, JonesTrading.

Matthew Cross: Following up on what Chad has said about 751, I was curious based on the results you've seen from 751 and the findings that you've demonstrated seem to indicate it has potential as a next-gen AKT. Can you comment on where you plan to continue development of 092?

I know at AACR, you presented some early in-vitro combination data with 092 in some cancer cell lines, but just wondering if you do expect this to remain more in the rare disease space with this candidate, and as you're working forward with 751? Or what your current thinking is at this point?

Paolo Pucci: We are planning to keep all of our options open -- that's the shortest answer -- until we can make a final decisions. So let's review what our options are and what will lead us to final decisions.

Our option for ARQ 092 miransertib, we have two parallel paths, the oncology path and the rare disease path. We hope that in the next few months, the cohort at Memorial Sloan Kettering is fully recruited for endometrial. And we hope that with that, we'll able to fully assess the data in that setting with the completed cohort. That would be an important element, too from our diseases. So we will continue with that effort in short.

For 092, for miransertib in rare disease, as I said, we are very convinced of what we have in proteus syndrome, and we hope to be able to assess the signal for PROS. So Overgrowth Diseases as of the data that should emerge from our company-sponsored Phase II trial. Then we will have to balance those two sets of data within miransertib alone because, obviously, the more the drug shines in rare diseases, the more our decision-making will lean toward that.

751 is introducing a next level of complexity, which is very welcome, because it's always good to know you have a drug and as we’re starting to see efficacy for the drug. As somebody wrote, the drug came in at AACR hoping to be a drug and came out of AACR knowing to be a drug. Then we will see what comes out of the conclusion of the Phase Ia trial for oncology to answer essentially the question for 751, does it have a better therapeutic window compared to the class and compared, obviously, to 092 in oncology?

So these are parallel processes that we hope to bring home sometime in the next few months and make the next round of a portfolio of decisions.

Brian Schwartz: As we discussed, we have evaluated preclinically combination options with immunotherapy, chemotherapy, and then our BTK inhibitor, so that we will have the

opportunity should we meet the go, no-go decision to move the agent into different settings as well.

Paolo Pucci: So, in short, we're thinking of portfolio approach to AKT. You almost want to look at the AKT franchise with the two assets as a portfolio, almost like a company onto itself, where you have a different set of prioritization within that individual company. Because the complexity there is significant; is rare disease; it is single agent oncology; and it is combination oncology as well. So that's the way we are approaching it.

And that's the reason why one of the rationales for us to partner derazantinib with Basilea because the level of complexity and the level of opportunities has increased exponentially here in the last, I would say, nine months.

Matthew Cross: I'm not sure how much you're able to give guidance on regarding the breakdown on the potential milestone payments for the derazantinib deal, but is this pretty heavily weighted towards completion of the Phase III? And I know there was a mention of a $3 million payment that could come from a predetermined milestone within the trial. Or would you say this is more triggered by commercial milestones?

Paolo Pucci: So, we cannot give, at this point in time, any more color than we have given in the press release. The mix of milestones, I would say, is the usual mix of milestones -- some are regulatory milestones, and some are going to be commercial milestones. And there is a small milestone that is related to passing the interim phase of the Phase III trial that is currently ongoing. And that's as much as I can comment.

Operator: (Operator Instructions)

George Zavoico, from B. Riley FBR.

George Zavoico: I'd like to focus a little bit more on the Basilea transaction or deal. How long do you think the transition will take for them to fully take control of conducting the Phase III trial? And is the plan to accelerate the iCCA trial perhaps by adding more sites, so that you can conclude the trial a little bit faster than 2020 as it says in the ClinicalTrials.gov filing?

Paolo Pucci: The answer to the first question is that there is provisions in the context that govern a few months of interactions, where we will continue to support and be reimbursed for those efforts the registrational trial. And of course, we would also support Basilea in any other way that they would desire intellectually, given that we have been in this FGFR space for quite some time and have accumulated quite a bit of knowledge.

But for your second question, I would have to defer you to the decision that Basilea will make. Basilea is a much larger company than we are. They're an experienced company in a very competitive field in the past and, the fact is and is now building into oncology.

And I'm sure that they will have updates for their investors along the way, and we will bounce those updates back, when relevant, in our calls.

George Zavoico: Will you maintain the Joint Steering Committee throughout the next several years or not?

Paolo Pucci: The answer is yes. There are the usual provisions for company interactions, which are important here because we have a third partner, which is Roivant, in the Greater China area.

George Zavoico: And looking at the Basilea pipeline, the derazantinib becomes their lead compound. From what I saw, they have a couple of products in Phase I or late Phase I. So this becomes their starship product in the spotlight, which suggests to me that they can be very committed to do this. But are there any other provisions in the contract to -- in case they don't, for example, commit to expand until their indications really get the product back?

Paolo Pucci: The contracts have all the use of provisions, but I wouldn't comment further because our mindset right now is to make this collaboration successful rather than otherwise. So our focus has been, how do we affect a seamless transition and in everybody's interests? For the rest, you can imagine that there are the standard provisions, as there are in any contracts. We have had such partnerships before -- I point you to the one we had with Daiichi Sankyo and Kyowa Hakko Kirin -- not much different (inaudible), George.

George Zavoico: And finally, looking at Basilea's sales infrastructure, they -- at least with some of their anti-infectives, they've outlicensed to several territories. Can you comment at all about -- and maybe it's too early to tell, what their sales plans might be in terms of sublicensing derazantinib once, hopefully, it successfully completes development and is approved?

Paolo Pucci: No, I can't, George. Because as before, for these round of questions and any future rounds, I'll have to refer to Basilea, who's the new steward of the project. What I can tell you is that there was interest in the drug, both globally and regionally. How would they pursue any of that interest that has remained unfulfilled, that I don't know.

Operator: I'm showing no further questions at this time. I would now like to turn the conference back to Peter Lawrence for any closing remarks.

Peter Lawrence: Thank you, Operator. Thank you, everyone, for your participation today. We look forward to speaking with you on our Q1 call on May 7. Goodbye.