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Alexion Pharmaceuticals Inc (ALXN) CEO Ludwig Hantson on Q3 2019 Results - Earnings Call Transcript

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Q3: 10-23-19 Earnings Summary

Press Release *Slides*

EPS of $2.79 beats by $0.31 | Revenue of $1.26B (23.05% Y/Y) beats by $25.13M

Earning Call Audio

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Alexion Pharmaceuticals Inc (NASDAQ:ALXN) Q3 2019 Results Earnings Conference Call October 23, 2019 8:00 AM ET

Company Participants

Susan Altschuller - Vice President, Investor Relations

Ludwig Hantson - CEO

Paul Clancy - Chief Financial Officer

John Orloff - Global Head of R&D

Brian Goff - Chief Commercial Officer

Aradhana Sarin - Chief Strategy and Business Officer and Incoming CFO

Conference Call Participants

Josh Schimmer - Evercore ISI

Matthew Harrison - Morgan Stanley

Geoffrey Porges - SVB Leerink

Chris Raymond - Piper Jaffray

Phil Nadeau - Cowen & Company

Geoff Meacham - Bank of America

Paul Matteis - Stifel

Mohit Bansal - Citi

Salveen Richter - Goldman Sachs

Laura Chico - Wedbush Securities

Rahul Prasad - William Blair

Operator

Ladies and gentlemen, thank you for standing by and welcome to the Alexion Pharmaceuticals Third Quarter 2019 Results Conference Call. At this time, all participant lines are in a listen-only mode. After the speakers' presentation, there will be a question-and-answer session. [Operator Instructions]. Please be advised that today's conference may be recorded. [Operator Instructions].

I would now like to hand the conference over to Susan Altschuller. Please go ahead, ma'am.

Susan Altschuller

Thank you and good morning. Thank you for joining us today for Alexion's performance in the third quarter 2019. Today's call will be led by Ludwig Hantson, our CEO. Ludwig will be joined by Paul Clancy, our Chief Financial Officer; John Orloff, our Global Head of R&D; and Brian Goff, our Chief Commercial Officer. Aradhana Sarin, our Chief Strategy and Business Officer and Incoming CFO will be available for Q&A.

You can access the webcast slides that will be presented on this call by going to the Events section of our Investor Relations page on our website. Before we begin, I would like to point out that we will be making forward-looking statements, and these statements involve certain risks and uncertainties that could cause our actual results to differ materially. Please take a look at the risk factors discussed in our SEC filings for additional detail. These forward-looking statements apply only as of today, and we undertake no duty to update any of the statements after the call, except as required by law.

I'd also like to remind you that we will be using non-GAAP financial measures, which we believe provide useful information for the understanding of our ongoing business performance. Reconciliations of our financial results and financial guidance are included in our press release. These non-GAAP financial measures should be considered in addition to, but not a substitute for, our GAAP results. Thank you. Ludwig?

Ludwig Hantson

Thank you, Susan, and good morning, everyone. I'm very pleased to share the outstanding performance in the third quarter and the continued progress of Alexion 2.0. I’m proud of the excellent execution across all aspects of our business. For the second quarter in a row we received multiple regulatory approvals, including most recently ULTOMIRIS for aHUS in the U.S. and SOLIRIS for NMOSD in the EU. These are in addition to the ULTOMIRIS PNH approval in the EU at the start of July.

Collectively, we have received five global regulatory approvals in the last four months and all launches are off to a strong start. We have also made significant progress further growing and diversifying our clinical stage pipeline with three business development deals; Eidos, Stealth and Achillion and we expect to add four new clinical stage assets, including two in Phase 3 developments. We are continuing to make remarkable progress evolving the company.

Slide 6 highlights key third quarter achievements beyond those I’ve just mentioned. First, our team has continued to demonstrate launch excellence across the globe, driving rapid PNH conversion to ULTOMIRIS. The U.S. launch trajectory continues upwards and the conversions in Germany and Japan are outpacing the rapid start we saw in the U.S.

Second, we continue to accelerate our neurology portfolio. Today is the second anniversary of the FDA approval of SOLIRIS for gMG, marking the company's entrance into rare neurology. Now just two years later, neurology is on track to be our largest franchise, driven by continued strong adoption in gMG as well as robust early demand in NMOSD.

Third, we continue to grow our metabolics portfolio with new STRENSIQ and KANUMA patients while also progressing the Phase 3 study of 1840 in Wilson Disease.

Fourth, we have further built our pipeline through both internal and external efforts. In addition to the new business development additions I highlighted earlier, we have also grown our pipeline by progressing our internal assets, including late stage with multiple registrational programs underway and even we’re on track to start in 2020, as well as early stage moving 1720 into the clinic.

And fifth, we have once again delivered on our financial ambitions, with strong revenue and volume growth this quarter.

As we look to the future, we're focused on the durability of our core franchises, as well as the further diversification of the portfolio to drive continued near and long-term growth. In order to do this, we're focused on three key priorities highlighted on Slide 7. The first is conversion. We believe that ULTOMIRIS is the future of C5 inhibition and the patient demand we see supports this.

Second is expansion. In particular of our C5 franchise there is an opportunity to help even more patients with ULTOMIRIS. And we have expanded the development program to seven diseases. PNH, HUS, gMG, NMOSD, HSCT-TMA, ALS, PPMS and also weekly subcu dosing. In addition, 1720 moved into the clinic. With both 1810 and 1720, we have optionality and the potential to treat a variety of complement-mediated diseases not addressed by our current portfolio.

A third area of focus is diversification, which we have specifically focused on as we have grown our pipeline. Much of this is driven by our ongoing business development efforts, which have included 11 deals in less than two years, seven of which have been clinical

stage and we continue to look for additional opportunities. We are rebuilding our pipeline from the ground up. Just two years ago, we had only three programs in late-stage clinical development: ULTOMIRIS for PNH; and aHUS; and SOLIRIS for NMOSD.

As you can see on Slide 8, our significantly expanded pipeline is poised to deliver at least 10 potential launches over the coming years. These launches span a range of disease areas, patient populations, routes of administration and geographies. Our expanded portfolio positions us well to deliver long-term sustainability and value creation with many important catalysts in 2020 and 2021.

Transforming a company does not happen overnight. And I'm incredibly proud of how much we've accomplished in a short time, meeting, and in many cases exceeding expectations.

We have established a very strong foundation on which to continue building Alexion 2.0 and remain committed to further evolving and growing the company to ensure it is positioned to deliver well into the future.

With that, I will now turn the call over to Paul to discuss our third quarter financial results. Paul?

Paul Clancy

Thanks Ludwig. Starting with Slide 10, we reported third quarter total revenues of $1.263 billion, an increase of 23% year-over-year. This was driven by gMG, continued growth in the core business and ULTOMIRIS PNH conversion. Our non-GAAP operating margin was 57%, an expansion of 357 basis points, driven by top-line growth. Non-GAAP earnings per share was $2.79, a 38% growth year-over-year.

Moving to Slide 11, third quarter net product sales were driven by volume growth of 23% and partially by a price tailwind of 1%.

Turning to Slide 12, SOLIRIS revenue in the third quarter was $991 million with year-over-year volume growth of 11%, driven primarily by strength in the United States and Japan, which both include the growing contribution from our gMG business. SOLIRIS revenues were partially impacted by ULTOMIRIS conversion.

ULTOMIRIS revenue in the third quarter was $90 million, which now includes contributions from Europe and Japan. Recall, there's a benefit in the quarter that each patient starts ULTOMIRIS treatment due to the loading dose impact. Subsequent quarters are then impacted by the maintenance dose discount in extended every 8-week dosing interval. We expect this dynamic will create some revenue fluctuation on a quarter-over-quarter basis going forward for ULTOMIRIS. Recall with aHUS, we anticipate the dynamic maybe more pronounced given the even greater maintenance dose discount to SOLIRIS.

The total revenue for our C5 franchise, which includes SOLIRIS and ULTOMIRIS, grew 22% year-over-year across PNH atypically aHUS, gMG and NMOSD. Underlying volume growth in the PNH and aHUS business remained solid in the quarter, and we continue to expect high-single-digit underlying volume growth for this year.

Moving to Slide 13, STRENSIQ revenues for the third quarter were $154 million representing 36% revenue growth and 36% volume growth year-over-year. KANUMA revenues in the third quarter were $28 million representing 12% revenue growth and a 16% volume growth year-over-year.

Turning to the P&L on Slide 14, during the quarter non-GAAP R&D expense was $186 million or 15% of revenues. Non-GAAP SG&A expense was $260 million or 21% of revenues. The non-GAAP effective tax rate in the quarter was approximately 11%. The GAAP rate was approximately 13%. Both Q3 non-GAAP and GAAP taxes benefited by a change in estimate of our current and prior year foreign-derived intangible income deductions based on additional guidance provided by the U.S. Treasury Department related to U.S. tax reform.

We reported third quarter non-GAAP EPS of $2.79 growing 38% year-over-year, GAAP EPS was $2.08. We ended the third quarter with approximately $2.2 billion in cash and marketable securities. In the third quarter, we repurchased $335 million in shares, in accordance with our share stabilization plan to offset equity awards. We've completed share stabilization for all of 2019 and a portion of 2020.

This week, the Board has approved a new authorization for $1 billion as we've nearly completed the prior authorization. This is primarily earmarked for share stabilization for future years. Strategic deployment to further build the pipeline remains our primary focus

for capital deployment.

I'll now turn to Slide 15, for our updated financial guidance, which Aradhana and I jointly developed. We're guiding to total revenues between $4.860 billion to $4.890 billion, an increase from prior guidance. This represents 18% growth year-over-year at the midpoint of the range.

For the combination of SOLIRIS and ULTOMIRIS, our revenue guidance is $4.180 billion to $4.200 billion. This assumes continued momentum in gMG, incorporates our ongoing launch of NMOSD in the U.S., and the launch of ULTOMIRIS for PNH in the U.S., Europe and Japan.

Turning to metabolics, our revenue guidance is $680 million to $690 million. We estimate price will be a 2% headwind in 2019 and foreign exchange impact net of hedging is expected to represent an approximate $40 million headwind. GAAP operating margin is expected to be between 41% and 42% inclusive of restructuring and related expenses as well as upfront payments.

Non-GAAP operating margin is expected to be between 55% to 56% of revenue, unchanged from prior guidance. Non-GAAP R&D expense is expected to be 14% to 15% of revenues. We intend to further build and progress the pipeline and expect clinical program spend especially for late stage development to increase in 2020.

Non-GAAP SG&A spend is expected to be 21% to 22% of revenues for full year 2019, slightly increased from prior guidance as we support the numerous launches around the world. We expect the non-GAAP effective tax rate to be approximately 13%. GAAP earnings per share is expected to be between $8.58 and $8.78. Non-GAAP earnings per share is expected to be between $10.25 and $10.40. The midpoint of the non-GAAP earnings per share is approximately 30% growth year-over-year. This guidance excludes the financial impact of the recently announced agreement to require Achillion as it's not anticipated to close until the first half of 2020. We've delivered a strong quarter and we're well positioned to deliver on our 2019 goals.

I'll now turn the call over to John to provide an R&D update.

John Orloff

Thank you, Paul. Here on Slide 17 is our development portfolio, which has grown substantially this year. We now have 22 planned development programs. And as Ludwig mentioned earlier, this leads to the potential for at least 10 launches by 2023, with significant milestones in 2020 and 2021. This further diversification includes three business development deals announced in the past two months with Eidos, Stealth and Achillion.

I will also note that we entered into a asset purchase agreement, pending bankruptcy proceedings with Immune Pharma to acquire an anti-eotaxin-1 antibody for potential development in inflammatory diseases. So we continue to bolster our early stage pipeline as well.

Turning to Slide 18 and our key late-stage programs starting with ULTOMIRIS. We expect to have at least five ongoing registrational trials in 2020 including HSCT-TMA, gMG, NMOSD, ALS and weekly subcutaneous formulation for PNH and atypical aHUS. We also anticipate initiating trials in additional indications and expect to update you on our strategy to further expand ULTOMIRIS next year.

Moving to ALXN1840 for Wilson's Disease, we remain on track to complete enrollment in our Phase 3 trial in early 2020. 1840 has the potential to be highly differentiated from current treatments due to 10,000 fold higher affinity for copper, potentially improving liver function and neuropsychiatric symptoms and significant improvement in dosing. Following review with regulators, we will be moving forward with a registrational Phase 2/3 trial for CAEL-101 in AL light chain amyloidosis stratifying patients by Mayo Clinic stages II, IIIA and IIIB.

Turning to our emerging anti-FcRN portfolio. We remain on track to manufacturer sufficient supply for 1830, our lead anti-FcRN asset by year-end and plan to initiate a Phase 2 study in warm autoimmune hemolytic anemia early next year. We will also begin a Phase 2 study of subcutaneous 1830 in gMG in the second half of 2020, following generation of subcutaneous data in healthy volunteers.

In addition, we anticipate data from the SAD/MAD study of ABY-039, a potential best-in-class subcutaneous anti-FcRN we are developing with Affibody in the first half of 2020. On the right, you see the late-stage programs from our three recently announced business development deals. I will provide a little more detail on each of these in the coming slides.

Moving to Slide 19, which illustrates that across these late-stage programs and platforms, we have the opportunity to diversify and significantly expand the reach of our portfolio to many additional patients with significant unmet needs. We're able to leverage our ultra rare and rare disease capabilities for these patients, who have very limited treatment options. And this is only the beginning. For example with oral Factor D and FcRN, we see opportunity in numerous complement-mediated diseases, many that impact much larger populations than those we currently treat.

Moving to Slide 20. We announced last week that we entered into an agreement to acquire Achillion. Pending the satisfaction of customer and closing conditions and relevant regulatory approvals, the acquisition is expected to close in the first half of 2020. This acquisition would add two clinical stage oral Factor D inhibitors to our pipeline. Factor D is a critical control point for the complement’s alternative pathway and is implicated in numerous rare diseases with significant unmet needs. For example, in the areas of nephrology and ophthalmology.

Importantly, selective inhibition of the alternative pathway allows Lectin and Classical pathways to continue to fight infection. Therefore, Factor D inhibitors have the potential to advance the standard-of-care with oral administration for multiple rare complement-mediated diseases. And both 4471 and 5228 provide important opportunities to diversify our complement portfolio beyond C5.

We also recently announced an option to co-develop and commercialize a late stage therapy for mitochondrial diseases with Stealth Biotherapeutics, elamipretide is a novel potential first-in-class therapy that targets mitochondrial dysfunction, which is currently being evaluated in a Phase 3 study in people with Primary Mitochondrial Myopathy or PMM, a genetic mitochondrial disease. There are no therapies approved to treat PMM, which is characterized by debilitating skeletal muscle weakness, chronic fatigue and exercise intolerance.

Phase 2 results from Stealth's MMPOWER-2 study showed strong improvement in six minute walk test results among patients with low walking ability. These results informed the inclusion criteria and primary endpoints for the ongoing Phase 3 MMPOWER-3 study, from which topline results are expected in the first quarter of 2020.

In addition, to exercise the option provides the opportunity to treat patients with Barth Syndrome, Leber's Hereditary Optic Neuropathy and geographic atrophy associated with dry age-related macular degeneration. We believe elamipretide has the potential to meaningfully improve the treatment of mitochondrial-driven disease, and we look forward to the opportunity to work with our partners at Stealth to bring this treatment to patients.

Moving to Slide 22, and our agreement with Eidos Therapeutics to develop AG10 for transthyretin amyloidosis or ATTR in Japan. ATTR is a progressive fatal disease in which transthyretin can be destabilized and misfolded, leading to amyloid fibroid deposition in organs. The majority of patients present with either neuropathy, cardiomyopathy or both, and often go through a series of specialists and misdiagnoses before eventually receiving a diagnosis of TTR amyloidosis.

AG10 is currently in Phase 3 development for ATTR cardiomyopathy in the U.S. and EU after Phase 2 results successfully show greater than 90% average TTR stabilization at day 28 of treatment.

Alexion will be responsible for development in Japan, where we look forward to applying our expertise to bring AG10 to Japanese patients.

The R&D organization has grown significantly to support our extended pipeline. It is exciting that the effects of this are already starting to be seen. I'm incredibly proud of the progress this quarter and we'd like to thank the global R&D organization for their dedication to advancing and further building our pipeline and portfolio strategy.

With that, I'll turn the call over to Brian to provide a commercial update. Brian?

Brian Goff

Thank you, John. I'll start on Slide 24 with an update on ULTOMIRIS conversion, then move to our great progress in neurology and the continued growth of the metabolic franchise. Starting with ULTOMIRIS for PNH in the U.S., we continue to be very pleased with the launch in our efforts to facilitate patient conversion.

As of the beginning of this week, 51% of patients are on treatment with ULTOMIRIS. This is unprecedented progress for rare disease facilitated conversion in just 10 months of launch. As with any launch curve, we do not expect to continue on a linear trajectory as we now have a majority of PNH patients converted.

That said, we have some promising developments to help maintain our momentum. First, a permanent J-Code for reimbursement was implemented on October 1st, removing that as a barrier for prescribing. And second, we're starting to target a greater breadth of treaters. So we remain on a very solid path towards achieving our accelerated goal of best-in-class patient conversion of at least 70% by mid-2020 in the United States.

Moving to our European launch progress, starting with Germany where we launched in July, we are already at over 45% conversion, which is more than three months ahead of the U.S. launch at the same time point. This is tremendous progress and a credit to the strength of the ULTOMIRIS product profile, the local team in Germany and benefited by the concentrated treater base.

We've also launched in Austria, Denmark and Finland, providing access to ULTOMIRIS in other European countries as a priority. And we're working towards additional launches in the EU as we seek reimbursement agreements. In Japan, we received approval in June and while we were waiting for the National Health Insurance price listing in order to launch, we were actively engaged in efforts to improve physician awareness of ULTOMIRIS. We officially launched in September and while it's still early days, conversion is tracking ahead of the U.S. launch at the same time point.

Turning to Slide 25, we're pleased to now have FDA approval for ULTOMIRIS to treat atypical aHUS in adult and pediatric patients. Here we'll leverage the strength of our PNH launch experience to drive rapid atypical aHUS facilitated conversion in stable patients. And our goal is to continue to deliver towards a best-in-class launch. I'll highlight a few subtle differences in the patient dynamics specific to atypical aHUS.

First, duration of therapy varies depending on etiology and is in general shorter than that in PNH given the silent nature of the disease between TMA events. We hope that with the profile of ULTOMIRIS and its every eight week dosing, there will be potential to improve persistence and compliance given the significantly increased risk of a TMA event if a patient stops treatment. That said, for some shorter duration populations, for example, atypical aHUS in pregnancy, there may be patients that choose to remain on SOLIRIS.

Second, newly diagnosed patients often present in acute settings. So we'll be working to help start patients on ULTOMIRIS or facilitate rapid conversion of SOLIRIS initiated patients once they transition to the outpatient setting.

Finally, we launched ULTOMIRIS with the objective of a global sustainable pricing strategy. Given that the labeled SOLIRIS dose for atypical aHUS is higher than that in PNH, this translates to maintenance dosing at a roughly 33% discount to SOLIRIS annually. This dynamic has already resonated very well with both patients and payers. Our team is highly prepared and we look forward to updating you on our progress here.

Moving to Slide 26, I'd like to step back and provide a bigger picture view of how we see ULTOMIRIS conversion, providing the foundation for the durability of our C5 franchise, focusing on the four core SOLIRIS indications.

Our ambition is to deliver best-in-class facilitated conversion for patients across the portfolio, and we're off to a strong start with the launches already underway. We believe that the vast majority of patients will be treated with ULTOMIRIS before even the earliest potential biosimilar launch.

Furthermore, we plan to have a weekly subcutaneous formulation available to provide even more optionality for patients. So we believe the market will move to and remain on ULTOMIRIS, given its strong clinical profile, globally sustainable pricing strategy and compelling value proposition including 20 fewer infusions needed per year.

Consistent with the U.S. timeline shown here, we expect to see similar dynamics play out in Germany and Japan, which can be seen in the appendix.

Turning to Slide 27 with an update on our neurology franchise which continues to deliver significant growth. As you can see on the left, we ended the quarter with more than 1,500 gMG and NMOSD patients treated with SOLIRIS in the U.S. This was our strongest quarter to-date for gMG and the NMOSD launch is also off to a great start. We believe our footprint and learnings in gMG have helped to accelerate the early trajectory in NMOSD.

Furthermore, the efficacy of SOLIRIS in NMOSD is profound and resonates with physicians, patients and payers given the unpredictability and the devastating and often irreversible impact of even a single additional relapse.

In particular, data from the Phase 3 extension showing that all patients on SOLIRIS monotherapy remain relapse free through 144 weeks is highly compelling.

Turning to Slide 28 and our metabolics franchise, we reported third quarter STRENSIQ revenue of $154 million or 36% year-over-year growth, driven by volume. With our CALIPER age-adjusting lab testing initiative, we're working to increase awareness and adoption of the appropriate diagnostic ranges for HPP helping to support earlier diagnosis, particularly of pediatric patients.

We reported third quarter KANUMA revenue of $28 million or 12% year-over-year growth. Again, this was driven by volume. With both LAL-D and HPP we continue to identify and treat additional patients and look to expand our geographic reach.

Once again, the hard work, expertise and dedication of the commercial team is inspiring, and has enabled us to deliver on multiple fronts and bring hope to patients with numerous rare diseases.

I'll now turn the call back to Ludwig for closing comments. Ludwig?

Ludwig Hantson

Thank you, Brian. We have made significant progress in the third quarter, as highlighted on Slide 29. Thanks to the tremendous efforts of all of our employees. Alexion 2.0 is off to a great start with a core business that is performing better than ever before, as well as an expanded and diversified pipeline.

As you can see on Slide 30, we are focused on durability and long-term growth by driving facilitated conversion, expansion of our current medicines into new diseases and further diversification of our portfolio.

Our current portfolio spans across hematology, nephrology, neurology, metabolics and cardiology, and is poised to deliver at least 10 additional potential launches by 2023 which will significantly evolve the composition of our core business.

There are many exciting milestones in the next 12 to 24 months. In particular, including multiple INDs, new programs entering the clinic, and new pivotal programs, as well as several data readouts from ongoing programs and we're not stopping here.

We continue to look to add additional rare diseases to our portfolio through further disciplined business development and internal research efforts. We also maintain our unwavering focus on patients and our commitment to advancing our mission of delivering life changing therapies to people living with rare diseases.

With that, we will now open up the call to questions. Operator?

Question-and-Answer Session

Operator

[Operator Instructions]. Our first question comes from Josh Schimmer of Evercore ISI. Your line is open.

Josh Schimmer

Maybe a quick one on myasthenia gravis launch in the U.S. Given the care paradigm, I might have expected that most of your diagnosed patients would have come on to SOLIRIS fairly quickly. And we might have even started to see a plateauing effect by now. It doesn't seem to be the case. Maybe you can help us understand the market dynamics here and the implications for the sustainable growth of the myasthenia gravis franchise? Thanks.

Brian Goff

Hi, Josh. This is Brian. So I'll take that as a compliment. So thanks a lot. Yes, the launch is going well. And we are -- as I noted, we're at the 2-year anniversary mark for our entry into neurology. We said at the time, when we started at the end of 2017 that we did not expect a bolus. We knew it would be heavy lift in terms of education around the role of complement in the underlying pathophysiology of gMG and we've made great progress. And along the way, we also expanded our commercial team to make sure that we could balance both depth of prescribing as well as breadth. And we did that as well to prepare, of course, for the NMOSD launch. And I think what you're seeing is just continued progress towards that educational effort.

And again, I'll just say that this is a population where while we talk about being over 1,500 patients now in the U.S. for gMG, that we're still just at the, I guess the leading edge, you can say, of the population that could be our addressable market. And that's 6,000 to 8,000 on the severe end of gMG in the U.S. alone, with the broader population of 60,000 to 80,000. So that continued educational effort we believe will continue us on our expansion journey.

Aradhana Sarin

And just to clarify, at the end of September, we were over 1,500 patients across gMG and NMOSD.

Brian Goff

Yes.

Aradhana Sarin

And have continued to add more patients since then.

Brian Goff

That's right.

Aradhana Sarin

Next question?

Ludwig Hantson

I was going to say that Brian and the team are doing an excellent job. And we believe that this business is sustainable as we're focusing on the refractory population. We know there's a lot of competition on the clinical trial side, but we're focusing on the patient type that might be different than some other technologies are focusing on. So we're really, really pleased with the progress.

Operator

Our next question comes from Matthew Harrison with Morgan Stanley. Your line is open.

Matthew Harrison

I was hoping to just take a little bit more into AML and see what sort of metrics you could provide thereon. Launch uptake, how we should think about that relative to what you saw from the MG launch. And any other feedback as you -- especially as you think about potential competitors coming on next year?

Ludwig Hantson

Good morning, Matthew. With NMOSD, I would characterize it as seeing strong start. And we're still relatively early in the launch. I've communicated before that, again, this will be a significant educational lift for clinicians. One starting point is to make sure that it's clear to clinicians that this is not MS. And the reason that that's important is because they think about, oftentimes relapses in MS as being recoverable. And we have a very strong focus on the fact that every relapse matters in NMOSD. And oftentimes, as I noted, these relapses are unpredictable. They're devastating and very often irreversible. So that's really the beginning point. And the second is the role of complement, which is very different. And there's a lot of off-label Rituxan use that’s out in the NMOSD community.

So this is the first approved treatment for NMOSD and we're pretty emphatic that there's a sense of urgency that clinicians need. In the early days what we're seeing right now is there are clinicians who have had some patients that they have already thought about as

a candidate for SOLIRIS and so that's good. They're moving on to therapy. And now we're intensifying our educational efforts to make sure that they identify others who could make that transition from things like Rituxan or immunosuppressive therapy.

John Orloff

And I'll just add this is John, with regard to competitive data. Well, the majority of our patients were on background ISTs, about 25% were on no background IST. That is essentially monotherapy and they showed a 100% relapse free all the way up to three years.

Operator

Our next question comes from Geoffrey Porges of SVB Leerink.

Geoffrey Porges

Quickly -- and I'm not sure whether it's a question for Brian or perhaps Paul, but a lot of confusion about all the moving parts in terms of the transition. But could you give us some sense of whether you believe SOLIRIS can remain flat over the next two to three years, even as the ULTOMIRIS transition occurs? Because obviously SOLIRIS is still being driven by NMO and MG. So could you give us a sense of how we should be thinking about that?

And then on a related note, your transition slides for U.S., EU, Japan, et cetera are very helpful. But could you talk a little bit about how often drug exclusivity will play out when a biosimilar is available? Do you have any examples where orphan drug exclusivity for a protected indication has been sufficient to avoid erosion by a biosimilar that's already on the market for an alternate indication? So is that's what we're going to be facing in the '23, '24 timeframe?

Paul Clancy

Geoff, let me -- this is Paul, I'll try to take a little bit of a crack at the first part of that question and then Brian will kind of give a perspective on the orphan drug. I think you want to think about it with respect to on a geographic perspective. Our neurology business

is disproportionately and we expected probably to stay that way, U.S. and Japan.

And then, I think the way we think about the PNH and aHUS business around the world is converting to ULTOMIRIS. So, that's probably the guide posts that I'd give you, we're seeing SOLIRIS hanging in there now, because of the strength of gMG, which as we said before, by the end of this year, neurology will be the largest patient franchise in the United States.

So, we expect that to continue to grow. And then, over a longer period of time, obviously, we've got ULTOMIRIS that we're studying in both gMG and NMOSD, which we were a little bit biased here, but we think those are high-probability trials. So over a longer period of time, we kind of see the business completely moving to ULTOMIRIS.

Brian Goff

Yes, And Geoff, I'll take the second -- I'll take the second part on, this is Brian on ODD. We're not aware of an exact analog of the situation that we that we faced where there is rare disease or in this case ultra-rare disease, ODD a biosimilar. And then a generation-one to generation-two switch that has the kind of delta between the product profile of SOLIRIS, which at that time, will be 15-plus year old technology versus ULTOMIRIS.

So, we haven't seen that. We do believe that ODD provides a level of protection, but I think the more important side of this whole story is that we will have conversion that will be in such a way where patients will have had many multiple infusion experiences with ULTOMIRIS.

So, it becomes a part of their life and the feedback that we get from everybody from clinicians, from patients as well as from payers is very encouraging. And so, I feel very good about the strength of the ULTOMIRIS product profile. Even if there were the availability of, at some point, a biosimilar, I don't see a switchback dynamic and we're starting to hear that reflected by KOLs as well.

Ludwig Hantson

And from the regulatory perspective, with ODD protection they wouldn't be able to get that indication in their labeling and therefore, it wouldn't be able to promote of it.

Operator

Our next question comes from Chris Raymond of Piper Jaffray. Your line is open.

Chris Raymond

Thanks. And just maybe sort of a little strategic question with regard to all the deals you guys have done and sort of the pipeline backfill. So just noticing with Stealth and the Achillion deals that you guys kind of have the beginnings of a nice little ophthalmology portfolio and I know these aren't lead indications.

But you still kind of highlight them and talk about them. So clearly, it's been very successful moving in a neurology as an indication expansion, but should we maybe start thinking about ophthalmology is maybe another area that it's of interest here or is that just a byproduct of these two deals? Thanks.

John Orloff

Yes, I think from a strategic perspective, clearly we're focusing on rare diseases -- ultra-rare and rare diseases. We're focusing on medicines that can be transformative and ophthalmology for sure, it's within that range. If we believe that we can make an impact on the journey of our patients, we will.

So, I wouldn't look at this as a byproduct, but have we agreed on which path we're going to take strategically within this disease area, I would say maybe more discussions to come in the next months.

Paul Clancy

And I would just add with the Achillion deal, we're most excited about the opportunity for alternative indications that expands into new territory for us and that includes ophthalmology. With -- I think proof of science in terms of alternative pathway involvement in various ophthalmological diseases. And then, of course Stealth that has ongoing Phase 2 studies in geographic atrophy and AMD as well as hereditary optic neuropathy. I think gets us squarely in potentially in the ophthalmology space very soon.

Operator

Our next question comes from Phil Nadeau of Cowen & Company. Your line is open.

Phil Nadeau

Just a question on the subcutaneous version of ULTOMIRIS, could you remind us of the volume of the injection there, and how long it takes to administer? And then as we look forward to the launch, what percent of ULTOMIRIS do you think would transition to the subcu, The patients consider this a big benefit? And what will happen in future indications? We have to investigate the subcu in each future indication or do you expect conversion based just on the PNH, aHUS stated that you're generating now? Thanks.

Paul Clancy

So, with regard to the subcu program, we are using two West Gen I devices, which deliver 3.5 mills, it's delivered over 9 minutes. And again, the device is user friendly, applied to the body, they don't see the needle and it has been actually tested and commercialized with other products. So, we're very bullish on the device and we're in the middle of our subcu program now, which is nearing completion of enrollment and we should have data sometime in the second quarter of next year.

Ludwig Hantson

And the fact it’s already commercially available will facilitate the regulatory process so, which is an important step. And I'm really excited about the subcu optionality. So, Brian.

Brian Goff

Yes. Hi, Phil, it's a good question and it is one that we're excited about, we've talked about subcutaneous is providing options for patients it may be, though, as we continue to do more market research that it goes beyond that and there are, clearly, some patients that with the profile that we believe we can offer with ULTOMIRIS that may be the choice that they go for with subcutaneous, it could be a function of their lifestyle, how active they are? It could be a function of the distance from an infusion center, or just simply that it's minimally invasive, once weekly or so formulation versus an IV delivery. And so, that's one that we look forward to, as well as we continue on that conversion journey.

Ludwig Hantson

Yes, I think the subcu could be another critical component to address the durability, but more to come next year when we should be able to share you -- with you the Phase 3 data for PNH and eventually also aHUS. Okay, next question.

Operator

Our next question comes from Geoff Meacham of Bank of America. Your line is open.

Geoff Meacham

I just have a few. Brian, on ULTOMIRIS your conversion rates are strong, so far, as you guys get closer to 70%, what would you say are the remaining barriers across the geographies? And then, I understand the dosing differences between conversion PNH and aHUS. But are there access or payer differences between the two? And then bigger picture for Ludwig or Paul, with all the recent transactions, the pieces are definitely in place for diversification.

I just wanted to ask you how you characterize R&D capacity at this point, are there room for continued product specific deals, platform sort of technologies or maybe are we at the later part of the innings in terms of the step up in BD? Thank you.

Brian Goff

Yes, Geoff, it's Brian, I'll start then on the ULTOMIRIS question, and maybe I'll just take a moment to do a little mini victory lap, because I am really proud of the progress of the team has made. We're really pleased that we're well on track towards our goal of greater than -- 70% or greater by the middle part of 2020. And just as a reminder that is moving the goalpost earlier than we originally thought that's based on the progress.

And as you know there will be an ambition to go beyond the 70%, but it's interesting that not long ago the questions that I was asked all the time is, is our ambition to bold? Is it achievable? And here we are 10 months in the launch and we have the majority of patients already converted, which is, as I noted, that's unprecedented.

And by the way the simple math is that 51% of 70% means we're already three quarters of the way there in terms of our goal. So what we focus on from here on, to your question is a broader range of prescribers, these are oftentimes clinicians who have one or two PNH patients so that -- that requires more time and effort for the broader reach. The other thing that is a tailwind and it came earlier than is typical, is the availability now of a permanent J-Code. Usually that comes in a year, we achieved it at the beginning of this month, which is 10 months into launch and that's very important for clinician confidence of reimbursement.

And we believe that that will be particularly helpful as we move forward toward the next phase of the launch. So we feel really good about our progression that we'll be able to make. Again, not just in the U.S. but in Germany and Japan, which is actually tracking even faster than the U.S.

Paul Clancy

Geoff, this is Paul. And I'll take a crack at your question as it relates to R&D capacity and the connection with business development. A few perspective on -- from a financial resource allocation perspective, I think we welcome the upward pressure on R&D and that's how our planning is thinking about it, particularly as we go into 2020, we've alluded to that over this call as well as the last call, so I think we want to kind of welcome that if there is good pipeline builds. From a people perspective, no doubt the R&D organization is feeling kind of the added work that is going on and we do plan to kind of address that with additional hires and additional kind of keep building capability.

So, while that's not trivial but it is absolutely solvable. And then, I think what Aradhana has built in terms of business development organization connected with the opportunity set in rare diseases, I think there is a pretty strong opportunity set externally to kind of press on with respect to the strategy of kind of continuing to build the pipeline and continuing to build the clinical pipeline as well. John, Aradhana anything to add?

John Orloff

Yes. I would just say that we've certainly grown considerably this year to keep pace with the BD deals that have come in and the pipeline growth both internally and externally. And we're in a position really to tell a really good story to get of high-quality people in the organization and expand our expertise. So, that's ongoing now and it's going well.

Aradhana Sarin

Yes, I think the other part of your question was, will we look at any different type of transactions? And I think it will stay true to, again adding to what is a good strategic fit for us whether that's staying within the rare disease focus, focusing on clinical stage assets. But again, expanding our capabilities we have been sort of therapeutic area agnostic.

We have been technology-agnostic and will continue to be that way. Again, looking for sort of the best assets in our view that can solve unmet clinical needs.

Brian Goff

And Geoff, it's Brian, again I -- Susan reminded me that I missed one part of your question to about atypical HUS, and the payer feedback that's I expect that to be a very compelling discussion, they already have a lot of knowledge now of ULTOMIRIS, we've made great progress with PNH with over 80% of commercial covered lives. Now with a defined policy, the economic story for ULTOMIRIS is even stronger with a 33% discount versus SOLIRIS in the maintenance fees. And as I noted, we have the permanent J-Code which covers PNH and atypical HUS.

Operator

Our next question comes from Paul Matteis of Stifel. Your line is open.

Paul Matteis

I think I have two for Brian. So now that you're halfway through conversion in the U.S., Brian, I was wondering, have you noticed anything demographically that is different or unique about the patients -- the PNH patients who haven't switched from SOLIRIS? And

I'd be curious what the implications of this are, if any? And then secondarily, what's going on in Germany? Why do you think that's gone so well so far? And what's the read through there on to dynamics in other European countries? Thanks so much.

Brian Goff

Hey, Paul. So on the first one in -- as we move into, I call it the next phase of our launch, some of the dynamics that we're seeing you -- anybody would expect there is, in some cases, lower awareness on the part of the patients around ULTOMIRIS or they're not as connected perhaps with other patients through advocacy or the like. There also is the process we're still working through is the frequency of patient visits to clinicians varies pretty widely across PNH.

And so some of it is just waiting for either a visit, or they need to have a few visits to have that appropriate discussion with the clinician. And then, the third thing I would notice again, we've moved largely through the -- I call it, the higher-volume type clinicians. And when I say high volume, it's with quotes, because this is ultra-rare and now we're moving into sort of one-off clinicians.

They have one or two patients as I had noted that's mainly the U.S. dynamic. In Germany, first of all, we've been able to really amortize the learnings of launch from the U.S., that's a real advantage of the sequential progress of U.S. then Germany. And as I noted, Japan is going well. Additionally, one key difference in Germany, is that the prescribing treaters are more concentrated than in the U.S.

So, that's not a discredit to what the team in Germany has done, but I think it's aided them with some of their speed to conversion.

Operator

Our next question comes from Mohit Bansal of Citi. Your line is open.

Mohit Bansal

I would like to start with congratulating Aradhana for her new role and also a big thanks to Paul for all the help. You will definitely be missed. Now on the question side, you saw quite a big jump in the number of patients in neuro side, could you clarify, to what extent it was MG versus NMO? And our math actually suggests that neuro could already be contributing 40% or so to SOLIRIS U.S., making it the biggest already. Can you please confirm that too? Thank you.

Brian Goff

Yes, we -- so Mohit, this is Brian. And by the way, I'll congratulate Aradhana as well. We're really thrilled to be -- to have Aradhana in her new position. On neurology, we have not given the breakdown of gMG versus NMOSD. In fact, it's the same -- commercially, this is the same line and so we think about it as a dedicated franchise. What I have noted is we're really pleased with the progress that we see on both fronts. This was our best quarter to date that we've had on our two-year anniversary actually with gMG. So that part's going really well. And as I had noted, we continue to make excellent progress with educating clinicians. So now we've got good balance of both breadth and depth of prescribing in gMG. In NMOSD, we're just a few months in, but are also very pleased about how that's progressing, that's going to be a journey, very similar to gMG with not a bolus but linear progression. And we see that becoming a very meaningful part of the business going forward. And we have made the stated ambition that neurology by patient volume in the U.S. becomes our biggest franchise and we're well on track for that goal.

Operator

Our next question comes from Salveen Richter of Goldman Sachs. Your line is open.

Salveen Richter

Just maybe one on the pipeline. When we look to this proposed, the Achillion transaction. Could you comment on your prior work with SOLIRIS in C3G and just contrast it with this approach and read through?

John Orloff

Yes. There's only been anecdotal reports of the use of SOLIRIS in C3G and certainly, nephrology has been an area that we're evaluating now, multiple renal diseases that -- where complement plays a role and that includes terminal complement as well as upstream complement targets. And so, with the Achillion's program, clearly, they've got two Phase 2 studies that are ongoing, that will read out next year with promising proof of mechanism data in patients following 14 days of treatment where they showed consistent effects on various biomarkers of the complement pathway that leads us to be optimistic about the potential results as we look at the Phase 2 studies, which will be evaluating our renal biopsy scores as well as eGFR and proteinuria.

Ludwig Hantson

We take two more questions.

Operator

Our next question comes from Laura Chico of Wedbush Securities. Your line is open.

Laura Chico

I was wondering if you could step back to the theme of kind of the diversification efforts. And if you could talk a little bit about the strategy around the ALS program that you're anticipating starting soon here, just in terms of patient selection and trial design? And I guess related to that we also noticed a patent filing on C5 inhibitors and AMD and I know there have been prior studies with SOLIRIS in dry AMD, but just curious on your thoughts about the potential for ULTOMIRIS in AMD? Thank you.

Brian Goff

So, I'll take the first part of that anyway. With regard to ALS and we've had input from a number of key opinion leaders and we're seeking regulatory guidance on a program that would be registrational in nature that delivers on endpoints that would be satisfactory to the multiple major regions, including the U.S., Europe and Japan.

We do have a final study protocol, we will be engaging regulators shortly and I hope to begin that trial in the first half of next year, that includes both functional evaluation. So the ALSFRS, our rating scale as well as looking at survival given that their mortality is high and rapidly progressive. So we'll be looking at both of those. In addition to that, we'll be looking at other functional evaluations such as pulmonary capacity, forced vital capacity and slow vital capacity. Right now, until we have the final design approved by regulators, we can't really say much more than that.

Ludwig Hantson

That we were at the top of the hours. So we take one, one last question.

Operator

Our last question comes from Rahul Prasad of William Blair. Your line is open.

Rahul Prasad

Assuming the Achillion deal closes, can you just kind of discuss danicopan and how it relates -- how will it relate to the ULTOMIRIS conversion? Would you plan on kind of initiating studies with ULTOMIRIS as an add-on therapy? And then just one question on BD, do you have a threshold of debt capacity that you'd be willing to take on with deals? Thanks.

Brian Goff

So, with regard to the first part of that, as you know, they will be sharing their Phase 2 data fourth quarter with regard to add-on to SOLIRIS. And then, the plan is to proceed with the regulatory engagement, since the deal hasn't closed, we really can't comment specifically on the plans, but we are excited about the opportunities beyond PNH and in PNH, we think it's certainly as add-on, can play a role for the unmet need in a small subset of patients who continue to have extravascular hemolysis. However, this is a disease of intravascular hemolysis, which is served by C5 inhibition, LDH reduction being the biomarker that's most closely associated with reduction and the risk of thrombosis and only a small subset have that extravascular hemolysis that's clinically -- of clinical consequence.

But we are really excited about the opportunities outside of PNH, including C3G but as well as many in nephrology, ophthalmology, dermatology and other areas that allow us to expand our therapeutic footprint.

Aradhana Sarin

And in terms of, in terms of business development, we do have obviously substantial financial capacity. You've seen the transactions that we've done both from a licensing and partnering standpoint as well as from an M&A standpoint, which Wilson, Achillion and Syntimmune transactions. We are right now, under one times leverage. So we do have substantial capacity. We really focus less on sort of what our threshold capacity is but really our ability to create value from the acquisitions that we do, and the greater the transaction size, obviously, we want to make sure that those transactions are value creating.

Ludwig Hantson

This is going to be it for today. As was already mentioned on the call, a big thank you to Paul. Paul was instrumental in transforming this company and he will continue to work with us in the next -- for the next nine months. Paul, thank you. A big welcome to Aradhana, great to have you as our new CFO.

As you saw, it was a great quarter and I really want to thank our employees for their hard work, the organization is on an exciting journey, we're transforming our business and thank you for your time and enjoy the rest of your day. Thank you.

Operator

Ladies and gentlemen, this concludes today’s conference call. Thank you for participating. You may now disconnect.