**Horizon Pharma Plc**
**Third-Quarter 2017 Conference Call**
**Nov. 6, 2017**

**Tina Ventura**
**Senior Vice President, Investor Relations**

Thank you, Aiyala. Good morning everyone and thank you for joining us.

On the call with me today are:

- **Tim Walbert**, Chairman, President and Chief Executive Officer;
- **Paul Hoelscher**, Executive Vice President, Chief Financial Officer;
- **Bob Carey**, Executive Vice President, Chief Business Officer;
- **Dr. Jeff Sherman**, Executive Vice President, Research & Development and Chief Medical Officer;
- **Dave Happel**, Executive Vice President, Commercial Development and Strategy;
- **Eric Mosbrooker**, Senior Vice President, Orphan Business Unit;
- **Vikram Karnani**, Senior Vice President, Rheumatology Business Unit; and
- **George Hampton**, Executive Vice President, Primary Care Business Unit.

Tim will provide a high-level review of the third-quarter and an update on the business. Paul will provide additional detail on our financial performance and guidance and Jeff will provide a brief update on our clinical development programs for our rare disease medicines, including the recently initiated Phase 3 trial for teprotumumab. Tim will then provide closing remarks and we will take your questions.

As a reminder, during today's call we will be making certain forward-looking statements, including statements about financial projections, our business strategy, and the expected timing and impact of future events. These statements are subject to various risks that are described in our filings made with the Securities and Exchange Commission (SEC), including our annual report on Form 10-K for the year ended Dec. 31, 2016, subsequent quarterly reports on Form 10-Q and our earnings news release, which was issued this morning.

You are cautioned not to place undue reliance on these forward-looking statements and Horizon disclaims any obligation to update such statements.

In addition, on today's conference call, non-GAAP financial measures will be used. These non-GAAP financial measures are reconciled with the comparable GAAP financial measures in our earnings news release and other documents from today that are available on our investor Web site at www.horizonpharma.com.

We have also posted an investor presentation to our website that summarizes our third-quarter results.

I will now turn the call over to Tim.

**Tim Walbert**
**Chairman, President and Chief Executive Officer**

Thank you, Tina, and good morning everyone.

This morning we announced another quarter of strong performance, driven by continued growth of our Orphan and Rheumatology business units. Third-quarter net sales were $271.6 million and adjusted EBITDA was $108.1 million. As we noted in our release, based on our year-to-date performance, we are raising our full-year 2017 net sales guidance range to $1.030 to $1.050 billion and raising the lower end of our full-year 2017 adjusted EBITDA guidance range to $350 to $375 million. Paul will cover our financial performance and guidance in greater detail shortly.

Before I review our third-quarter business unit results, let me briefly comment on our long-term strategic direction and where we are in that journey as a company. For those of you that have followed us, you know that Horizon Pharma is a very different company than we were when we first launched as a public company in 2011. At that time, we were focused on building out our infrastructure and commercial footprint to generate sustainable earnings and cash flow. We did that quite quickly via our Primary Care business, and in the second half of 2014, we began the next phase of our strategy, which focused on rapid diversification of our business into rare diseases. Over the last three years, beginning with our acquisition of Vidara in September 2014 and subsequent acquisitions of Hyperion, Crealta and Raptor, we rapidly built a rare disease business that now represents the majority of our total company net sales – about 60 percent today – and sales of those rare disease medicines increased 65 percent year over year in the third quarter.

Today, we are focused on the growth of our rare disease medicines – which include our durable growth medicines RAVICTI®, PROCYSBI® and ACTIMMUNE® – as well as our high-growth biologic, KRYSTEXXA®. Our significant progress in revitalizing and repositioning KRYSTEXXA, as well as our recent expansion into the nephrology space, led us to raise our peak sales expectations to more than $400 million earlier this year, and today we provided our expectation that KRYSTEXXA net sales will grow more than 50 percent in 2018. This assumes that the 340B ceiling drug price will go into effect on July 1, 2018.

Commercial execution is a key competency for the company. But sustainable growth over the long term requires more than that. It requires a pipeline of differentiated and clinically relevant development-stage medicines, which we are now building. The acquisition of teprotumumab on May 8 marked the beginning of this important next phase of our strategy, and we have a uniquely strong business development team making a significant effort to build and acquire a portfolio of development-stage clinical candidates.

On Oct. 25, ahead of our original expectations, we announced the first patient was enrolled and infused in our Phase 3 confirmatory clinical trial evaluating teprotumumab for the treatment of thyroid eye disease, or TED. TED is a rare, painful and debilitating condition in which the eye muscles and fatty tissue behind the eye become inflamed. This often causes proptosis, where the eyes are pushed forward causing the eyeball to protrude from the socket, which can impair full eyelid closure and cause corneal ulceration and other serious complications. Teprotumumab demonstrated groundbreaking efficacy in its Phase 2 clinical trial, the results of which were published in *The New England Journal of Medicine* in May this year. Jeff will walk through these Phase 2 results in a moment as well as an update on this newly initiated Phase 3 trial.

The dramatic Phase 2 results, along with recent interactions with the Phase 3 investigators, have significantly increased our confidence in enrolling the Phase 3 trial in a timely manner. Furthermore, we are completing our teprotumumab market research and commercial planning for the U.S. and ex-U.S. markets, and preliminarily, our assessments show teprotumumab's net sales potential increasing considerably.

I will now review our third-quarter business unit results.

**Orphan Business Unit Third-Quarter Net Sales**
First, our orphan business unit generated $117.4 million in net sales in the quarter, an increase of 64 percent year-over-year.

RAVICTI net sales for the quarter increased 21 percent year-over-year to $50.9 million. This was driven by continued conversion of patients from older-generation nitrogen-scavenger therapies as well as an increase in treatment-naïve patients. Active shipping patients increased more than 25 percent in the third quarter compared to last year. We continue to expect double-digit net sales growth for RAVICTI in 2017.

PROCYSBI net sales in the quarter were $33.5 million, which no longer include net sales in the Europe, Middle East and Africa regions following our sale of these rights to Chiesi at the end of June this year. At the time of divestiture, we stated the sale would result in a $15 million reduction of PROCYSBI and QUINSAIR net sales in the second half, with the majority of affected sales coming from PROCYSBI. Additionally, PROCYSBI was launched in Canada at the end of October, where it is the only cystine-depleting medicine approved in Canada for the treatment of nephropathic cystinosis.

Third-quarter net sales for ACTIMMUNE were $29.2 million, an increase of 17 percent compared to the third quarter of 2016. Through the first nine months, ACTIMMUNE net sales are up 5 percent year over year, and we continue to expect a similar rate of growth for the full year.

**Rheumatology Business Unit Third-Quarter Net Sales**
In our Rheumatology business unit, which includes both KRYSTEXXA and RAYOS, third-quarter 2017 net sales increased 44 percent to $58.1 million.

KRYSTEXXA generated net sales of $42.8 million, an increase of 67 percent compared to the third quarter of 2016, and this was primarily driven by strong KRYSTEXXA vial growth. We expect continued strong demand for KRYSTEXXA moving forward, particularly given the commercial and clinical investments we are making in this medicine. And as I mentioned earlier, we expect net sales of KRYSTEXXA to grow greater than 50 percent in 2018.

As of the fourth quarter, we are targeting nephrologists for the first time. In addition to the large, hard tophi that occur on fingers, toes and elbows, chronic gout patients also have many co-morbid conditions. Gout does not just occur in the joints; uric acid deposits can also build up in the organs, such as the heart and kidneys. In fact, gout is highly correlated with chronic kidney disease, or CKD – between 25 to 50 percent of CKD patients have gout. Our data indicates that there are approximately 50,000 uncontrolled gout patients currently being treated by nephrologists, providing a significant opportunity to accelerate the number of patients treated with KRYSTEXXA. We now estimate the total addressable patient population for uncontrolled gout patients treated by both rheumatologists and nephrologists to be approximately 100,000 in the United States. This year, we estimate there are between 1,500 and 2,000 uncontrolled gout

patients being treated with KRYSTEXXA, representing less than 2 percent of the addressable market. We expect to rapidly and substantially grow our share of this market over the coming years.

Building on the strong commercial team we put in place last year, we began a second expansion in the third quarter. We have hired highly experienced biologic sales specialists, as well as medical scientific liaisons and patient access managers to further accelerate the number of patients receiving the benefit of treatment with KRYSTEXXA. We expect to complete the expansion in the fourth quarter. Our expanded commercial team is embarking on new initiatives to further penetrate the current rheumatology audience and, over the last few weeks, began promoting KRYSTEXXA to nephrologists. We therefore expect our expanded commercial organization to be fully trained and begin to drive new patients treated as we move into 2018. Early feedback has been positive with a number of patients already being treated in nephrology.

In addition to our commercial investments, we continue to educate clinicians about the compelling efficacy and safety profile of KRYSTEXXA. We had a significant presence at the American Society of Nephrology meeting last week and the American College of Rheumatology meeting going on this week, and Jeff will discuss the KRYSTEXXA data being presented at both of these meetings.

**Primary Care Unit Third-Quarter Net Sales**

In our Primary Care business, third-quarter net sales were $96.1 million, which is in line with our expectations.

With that, I will now turn the call over to Paul.

**Paul Hoelscher**
**Executive Vice President, Chief Financial Officer**

Thanks, Tim.

My comments this morning will primarily focus on our non-GAAP results, unless otherwise indicated.

**Third-Quarter 2017 Financial Results**
Net sales totaled $271.6 million, driven by continued strong growth in the Company's Orphan and Rheumatology business units.

Our non-GAAP gross profit percentage was 89.6 percent in the third quarter, in line with our expectations.

Total non-GAAP operating expenses were $135.6 million, which was somewhat lower than our expectations, as the timing of some spend shifted from the third quarter to the fourth quarter.

Non-GAAP R&D expense was $17.0 million, primarily driven by preparation for the Phase 3 trial of teprotumumab, as well as continued clinical investments in KRYSTEXXA and RAVICTI.

Non-GAAP SG&A expenses were $118.6 million. The increase versus the third quarter of 2016 was primarily due to expanded commercial investments in KRYSTEXXA and expenses related to the Raptor business we acquired in October of 2016.

Third-quarter 2017 adjusted EBITDA was $108.1 million.

The non-GAAP income tax rate for the third quarter of 2017 was 47.3 percent, in line with our expectations.

Non-GAAP net income and non-GAAP diluted earnings per share in the third quarter of 2017 were $43.1 million and 26 cents, respectively.

The weighted average diluted shares outstanding used to calculate non-GAAP diluted earnings per share for the third quarter of 2017 were 165.8 million shares.

Our GAAP operating cash flow was $68.3 million, and non-GAAP operating cash flow was $83.5 million. At Sept. 30, cash and cash equivalents were $625 million.

The total principal amount of our outstanding debt as of Sept. 30 was $2.023 billion, and net debt was $1.398 billion. Our net debt to last-12-months adjusted EBITDA leverage ratio was 3.3 times. Based on our current guidance and cash generation expectations, we expect our net debt to adjusted EBITDA leverage ratio at year-end to remain below 4 times, assuming no additional M&A activity this year.

On Oct. 23, we refinanced our senior secured term loan at an interest rate of LIBOR plus 3.25 percent, a 50-basis-point-reduction compared to the previous interest rate of LIBOR plus 3.75 percent. Our current capital structure results in a weighted-average cash interest rate of 5.2 percent based on current LIBOR rates.

**Full-Year 2017 Guidance**
This morning we increased our full-year 2017 net sales guidance range to $1.030 billion to $1.050 billion from $1.010 billion to $1.045 billion, and raised the lower end of our full-year 2017 adjusted EBITDA guidance range to $350 million to $375 million from the previous $340 million to $375 million.

Our revised net sales guidance incorporates the following assumptions:

- Full-year net sales percentage growth for the Orphan business unit in the mid-50s, which is unchanged from last quarter;
- Full-year net sales percentage growth for the Rheumatology business unit in the mid-40s, which is an increase from our previous expectation of mid-30s growth, related to the delay of the implementation of the 340B drug ceiling price rule to July 1 of 2018; and
- Full-year net sales for the Primary Care business unit to exceed $350 million, which is unchanged from last quarter.

We continue to expect that KRYSTEXXA will generate strong net sales growth this year, next year and beyond. This is reflected in the 2018 guidance we provided today for KRYSTEXXA net sales growth of more than 50 percent, which also includes the estimated impact of the potential implementation of the 340B drug ceiling price rule on July 1, 2018.

We continue to expect full-year non-GAAP gross margin ratio to be approximately 89 to 90 percent.

We continue to expect second-half non-GAAP operating expenses to be modestly higher than the first half, with some spend moving from the third quarter to the fourth quarter due to timing, some related to our KRYSTEXXA investment. We also expect a higher level of R&D investment in the fourth quarter driven by the teprotumumab Phase 3 trial, which began enrolling patients in October, as well as our continued investment in investigator-initiated clinical trials for KRYSTEXXA.

We expect full-year net interest expense to be approximately $105 million, based on current LIBOR rates.

We continue to expect a non-GAAP tax rate for full-year 2017 in the low 30s, and we continue to project our full-year 2017 cash tax rate to be in the low single digits.

Our full-year 2017 weighted average diluted share count is expected to be roughly 165 million shares.

With that, I will now turn the call over to Jeff.

**Jeff Sherman, M.D.**
**Executive Vice President, Research & Development and Chief Medical Officer**

Thank you, Paul.

**Teprotumumab**
I will begin with teprotumumab, our fully human monoclonal antibody in Phase 3 development for the treatment of moderate-to-severe TED. There are no FDA-approved therapies for TED, and thus there is a significant unmet need for an effective and safe treatment. Because there are no approved therapies, the currently used options, such as high-dose steroids, result in limited efficacy and frequent safety issues. Often, the only option for TED patients is surgery, which is highly complex, invasive, and may only result in partial effect. Furthermore, it often needs to be repeated multiple times given technical challenges in this disease. In fact, many patients may have three or more surgeries per eye.

Teprotumumab is an insulin-like growth factor-1 receptor inhibitor, or IGF-1R inhibitor, and works by blocking the specific autoimmune pathophysiology that causes active TED. By blocking IGF-1R, teprotumumab diminishes local inflammation, prevents orbital fibroblast proliferation and reduces tissue expansion, thus restoring the orbital tissue to a more normal state. The groundbreaking Phase 2 results published in *The New England Journal of Medicine* in May describe teprotumumab as having a potentially disease-modifying treatment effect.

The Phase 2 trial showed that, for the intent-to-treat study population, 69 percent of study patients receiving teprotumumab demonstrated a statistically significant response compared to 20 percent of patients in the placebo group at week 24, with a p value greater or equal to 0.001. The primary endpoint of the Phase 2 trial was the response in treatment of the study eye, defined as a reduction in the Clinical Activity Score of 2 points or more and a reduction of proptosis of 2 millimeters or greater at 24 weeks. These results have generated a great deal of excitement on the part of physicians and patients as the development of teprotumumab advances.

Reflecting this excitement, on Oct. 25, we announced the first patient was enrolled in our Phase 3 confirmatory trial, named OPTIC, ahead of schedule. Seventy-six patients in total will be enrolled in the study. The primary endpoint of the Phase 3 trial, as agreed to with the FDA, is the effect of teprotumumab versus placebo on the proptosis responder rate at Week 24. This is defined as the percentage of participants with a reduction of more than or equal to 2 millimeters from baseline in the study eye, which is a readily measurable and objective endpoint. The main secondary endpoint is the same as the primary endpoint of the Phase 2 trial.

We expect to be in a position to submit data from the Phase 3 trial in the second half of 2019. Teprotumumab has U.S. FDA Orphan, Fast-Track and Breakthrough Therapy designations, which could allow for a six-month review timeline, if granted by FDA.

Teprotumumab exemplifies our focus to acquire development-stage medicines through our business development efforts so that we can bring highly differentiated and clinically compelling therapies to patients living with diseases that have limited treatment options. An additional component of our strategy is to collaborate with leading academic institutions and key opinion leaders to optimize our currently marketed medicines through further scientific study. This includes work underway with KRYSTEXXA.

**KRYSTEXXA**

There are four KRYSTEXXA posters being presented at this week's American College of Rheumatology Annual Meeting, and we had one at last week's American Society of Nephrology Kidney Week Meeting.

Emerging data from the ongoing, investigator-initiated TRIPLE trial will be presented later today at the ACR meeting by Drs. Ken Saag and Peter Lipsky. The trial is adaptive in nature and designed to answer a number of questions about KRYSTEXXA, including how to improve the response rate to KRYSTEXXA and reduce infusion reactions. Of note, this study is the first to demonstrate prospectively that when treatment "stopping rules" are followed, which is occurring more frequently in real-world practice, the rate of infusion reactions can be dramatically reduced, with the rate being less than one percent to date in the ongoing TRIPLE trial versus 26 percent in the current KRYSTEXXA label. We have submitted a safety update to the FDA in which we have proposed an update to the prescribing information for KRYTEXXA. This submission is based on additional analysis of the Phase 3 clinical trials, post-marketing safety data, and supported by data to date from the TRIPLE trial that collectively demonstrated a very low infusion reaction rate when stopping rules were used.

As the TRIPLE trial continues to evolve and progress, we expect it to generate additional data and subpopulation analyses that will help the gout community continue to learn how to treat patients more effectively. Dr. Lipsky is adding more cohorts, including one evaluating if the addition of a commonly used immunomodulator, azathioprine, has the potential to improve response rate. TRIPLE is one part of a broader, comprehensive strategy that we have in place to evaluate ways to improve the response rate to KRYSTEXXA. Another investigator-initiated trial is being conducted by Dr. Ken Saag at the University of Alabama Birmingham. The trial is named RECIPE and will evaluate immunomodulation with mycophenolate treatment along with KRYSTEXXA. The RECIPE trial is expected to begin by the end of the year.

Several other KRYSTEXXA abstracts are also being presented at the American College of Rheumatology meeting:

- This includes data that shows responders to KRYSTEXXA experienced significant reductions in blood pressure, independent of changes in renal function. This is an important finding as we evolve our understanding of KRYSTEXXA in the nephrology area and in chronic kidney disease patients. As Tim mentioned, chronic kidney disease is a frequent co-morbid condition for patients with uncontrolled gout. We presented this blood pressure data at the American Society of Nephrology meeting as well.
- An additional poster concluded that the lower the serum uric acid levels achieved with KRYSTEXXA, the faster a patient's tophi resolve; and
- A final poster assesses evidence-based development of criteria for complete response in patients with uncontrolled gout using KRYSTEXXA Phase 3 study data.

As noted in our earnings news release, we will be discussing this information in greater detail on a KRYSTEXXA-focused investor call later this week.

**PROCYSBI**

Data on PROCYSBI was also presented at last week's American Society of Nephrology meeting. The study demonstrated the impact of one year of PROCYSBI therapy on children six years of age or younger with nephropathic cystinosis who had never before received cysteamine treatment. The children in the study were able, with PROCYSBI, to maintain their cystine levels – a biomarker for disease control – and

also to reach several important physical development milestones, such as height, weight and body surface area, similar to what are expected for an average child of the same age.

**ACTIMMUNE**

Finally, I will briefly update the work on three cancer-combination studies with ACTIMMUNE, which continue to progress.

An investigator-initiated study at the Moffitt Cancer Center is underway and enrolling patients. The study is evaluating ACTIMMUNE in combination with Herceptin, Perjeta and Taxol and aims to determine the optimal dosing and treatment combination in certain advanced breast cancer patients.

The other two investigator-initiated combination therapy trials focus on PD-1 inhibitors. The National Cancer Institute-supported program evaluating ACTIMMUNE in combination with Keytruda to treat cutaneous T-cell lymphoma patients is on track to begin by year end. In addition, the Fox Chase study evaluating ACTIMMUNE in combination with Opdivo continues to enroll its fourth cohort of patients and we expect to have dose-level results by the end of the year.

I will now hand the call back to Tim for his final comments before Q&A.

**Tim Walbert**
**Chairman, President and Chief Executive Officer**

Thanks Jeff.

- We delivered another quarter of strong performance driven by continued growth of our Orphan and Rheumatology business units;
- Our rare disease medicines now represent 60 percent of sales and increased 65 percent in the quarter;
- We increased our full-year 2017 net sales guidance range to $1.030 billion to $1.050 billion and raised the lower end of our full-year 2017 adjusted EBITDA guidance range to $350 million to $375 million;
- We're delivering against our commercial priorities and are investing in KRYSTEXXA to generate sustainable, long-term growth, including more than 50 percent net sales growth expected in 2018; and
- we continued to make progress on the next phase of our company's strategy, which is to build a development-stage pipeline to drive our growth over the long term. We advanced this strategy with the initiation of the teprotumumab Phase 3 confirmatory trial last month, ahead of our original expectations.

At this point we'll open the call up for questions.

**Tina Ventura**
**Senior Vice President, Investor Relations**

Thank you, Aiyala.

Please mark your calendar for several upcoming events:

Later this week, on Thursday, Nov. 9 at 10 a.m. eastern time, we will hold an investor webcast on KRYSTEXXA, gout and our clinical strategy for KRYSTEXXA following the American College of Rheumatology meeting. We will have both Dr. Peter Lipsky and Dr. Ken Saag, who are leading the TRIPLE and RECIPE trials respectively, joining us for the webcast and Q&A. A live webcast and a replay may be accessed at the investor relations section of the website at www.horizonpharma.com.

We are also presenting at several investor conferences in the month of November, all of which will be webcast:

- The Stifel Healthcare Conference on Tuesday, Nov. 14;
- The Jefferies Autumn 2017 Global Healthcare Conference on Thursday, Nov. 16;
- The Piper Jaffray Healthcare Conference on Tuesday, Nov. 28; and
- The Bank of America Leveraged Finance Conference on Wednesday, Nov. 29.

That concludes our call this morning. A replay of this call will be available in approximately two hours by calling 1-855-859-2056 and the passcode for that replay is 96805714. Thank you for joining us today.