Horizon Pharma Plc
Fourth-Quarter 2017 Conference Call
February 28, 2018

Tina Ventura
Senior Vice President, Investor Relations

Thank you, James. 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;
- **Shao-Lee Lin, M.D., Ph.D.**, Executive Vice President, Head of Research and Development and Chief Scientific Officer;
- **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 fourth-quarter and full-year results and an update on the business, and Paul will provide detail on our financial performance and 2018 guidance. Shao-Lee will provide an update on our pipeline development programs. 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, including our annual report on Form 10-K for the year ended December 31, 2017, 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 Pharma 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 website at www.horizonpharma.com.

We have also posted an investor presentation to our website that summarizes our 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.

2017 capped off a year of significant progress for the company in which we launched the next stage of our strategy – building a pipeline of clinically meaningful medicines to drive long-term sustainable growth and shareholder value.

Our acquisition of teprotumumab for thyroid eye disease and the initiation of its Phase 3 trial marked the beginning of this important next phase. Following additional diligence on the patient population and market opportunity, in January, we raised our teprotumumab peak-year annual net sales guidance to more than $750 million.

Also in January, we named Shao-Lee Lin our new executive vice president, head of R&D and Chief Scientific Officer. Shao-Lee is a 20-year pharmaceutical executive, physician and scientist. Her arrival is a meaningful step in the Company's evolution as she will be leading our pipeline expansion initiatives.

In addition to teprotumumab, we added three new development programs to augment our rheumatology portfolio and enhance our leadership in the uncontrolled gout market. With these new programs in place, we are increasing our investment in R&D in 2018 and beyond, aligned with our strategy.

This morning we reported full-year 2017 total net sales of $1.056 billion and adjusted EBITDA of $389.7 million. Our fourth-quarter and full-year 2017 performance was driven by better-than-expected performance of our rare disease medicines, which increased 60 percent for the full year. Our rare disease medicines now comprise 60 percent of our total net sales, which underscores the value of the rapid transformation and diversification of our portfolio over the last few years.

We completed another impressive quarter and year for KRYSTEXXA®, generating full-year vial growth of 40 percent and completing the second expansion of our commercial organization. This expansion and the potential we see for thousands more uncontrolled gout patients to benefit from this medicine, led us in January to increase our KRYSTEXXA peak annual net sales estimate to more than $750 million.

As we noted in our earnings release this morning, we are significantly increasing our promotional and clinical investment in KRYSTEXXA in 2018 to drive our growth expectations for 2018 and future years. This includes our expectation this year for more than 50 percent year-over-year KRYSTEXXA net sales growth.

Since acquiring KRYSTEXXA in 2016, we have significantly changed the growth trajectory of this medicine by putting in place the right strategy, the right team and the right amount of investment.

We saw that begin to pay off in2018. Overall, our strong performance in 2017 resulted in robust cash flow generation that provides us with the financial flexibility to continue executing our strategy. Non-GAAP operating cash flow for the full year was $393.1 million; our year-end cash balance was $751.4 million; and our net debt to adjusted EBITDA leverage ratio remains at 3.3 times.

We are providing 2018 full-year net sales guidance of $1.15 to $1.18 billion, representing double-digit year-over-year growth at the midpoint and continued strong performance from our rheumatology and orphan business units.

We are providing 2018 full-year adjusted EBITDA guidance of $370 to $395 million, reflecting strong top-line growth as well as continued investments we are making in teprotumumab, our development pipeline and our key growth driver, KRYSTEXXA.

Paul will provide additional detail on our guidance and financial performance in a few minutes.

I will now briefly highlight our 2017 results by business unit.

Rheumatology Business Unit Net Sales
Net sales for our rheumatology business unit, which includes both KRYSTEXXA and RAYOS®, increased 48 percent for the fourth quarter and 50 percent for the full year.

KRYSTEXXA generated fourth-quarter net sales of $43.8 million, a year-over-year increase of 48 percent, and full-year net sales of $156.5 million, a year-over-year increase of 72 percent.

We completed the expansion of our KRYSTEXXA commercial organization in the fourth quarter, doubling the team to nearly 200, a move that will now allow us to reach more rheumatologists and expand our reach to nephrologists. Our expectation for strong growth in 2018 reflects the impact of this expansion, as well as the significant increase we've noted in prescriber conviction once they see the therapeutic benefits of KRYSTEXXA in their patients.

We continue to see significant opportunity to offer solutions for more patients with uncontrolled gout. Gout is a chronic, inflammatory, systemic disease most commonly associated with joint pain and visible tophi. However, uric acid deposits also build up in the organs, such as the heart and kidneys. There is increasing evidence to support the significant impact of chronically elevated uric acid on other body systems, including hypertension, diabetes and chronic kidney disease.

Our data indicates that there are approximately 50,000 uncontrolled gout patients currently treated by nephrologists, providing a significant opportunity to accelerate the number of patients treated with KRYSTEXXA – doubling the addressable patient population to approximately 100,000 in the United States. Given that we treated less than 2 percent of the addressable patient market in 2017, we see considerable opportunity with our recently expanded commercial team to rapidly and substantially grow our share of this market over the coming years. We are very pleased by the early positive feedback from nephrologists.

We continue to expect more than 50 percent year-over-year net sales growth for 2018, accelerating in the second half as the expanded commercial team hits its stride. Our growth projection also assumes the implementation of the 340B drug price rule on July 1st.

RAYOS net sales increased 38 percent for the fourth quarter to $15.6 million and 10 percent for the full year to $52.1 million.

Orphan Business Unit Net Sales
Our orphan business unit generated fourth-quarter net sales of $116.6 million, a year-over-year increase of 32 percent. Full-year 2017 net sales were $466.8 million, an increase of 56 percent. Strong growth of RAVICTI® and PROCYSBI® contributed to this performance, as did the October 2016 acquisition of PROCYSBI.

RAVICTI net sales for the fourth quarter increased 57 percent year-over-year to $51.9 million. Active shipping patients grew more than 20 percent compared to the fourth quarter of last year as we saw continued conversion from older-generation nitrogen-scavenger therapies and the addition of treatment-naïve patients. This growth was in part due to the April 2017 update of the RAVICTI label, which expanded the use of the medicine to patients older than two months of age from two years of age and older. Yesterday we submitted a supplemental new drug application (sNDA) to the FDA to expand the age range for UCD patients to birth and older from the current range of two months of age and older.

PROCYSBI net sales in the fourth quarter, which no longer include EMEA revenues following our sale of that business to Chiesi in June 2017, increased 31 percent to $33.2 million.

In December, we expanded the PROCYSBI label to include children one year of age and older, as well as to include new data we presented at the American Society of Nephrology meeting in November. This data is meaningful for children with nephropathic cystinosis. It demonstrates that children six years of age or younger, naïve to treatment, were able with PROCYSBI to maintain their cystine levels, which is a biomarker for disease control. Further, they experienced measured improvements in height, weight and body surface area, reaching important growth milestones equal to standard measures of unaffected children of the same age.

Fourth-quarter net sales for ACTIMMUNE® were $26.8 million, up 11 percent compared to the fourth quarter of 2016, which is driven by our continued efforts to establish the role of ACTIMMUNE in a broader range of chronic granulomatous disease patients.

Primary Care Business Unit Net Sales
In our primary care business, fourth-quarter net sales were $96.2 million, and full-year net sales were $375.4 million, both of which were 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.

Fourth Quarter and Full-Year 2017 Financial Results
Fourth-quarter net sales totaled $274.2 million, and full year net sales were $1.056 billion, driven by continued strong performance of our orphan and rheumatology business units.

Our non-GAAP gross profit ratio was 89.3 percent for the fourth quarter, and 89.6 percent for the full-year, in line with our guidance of 89 to 90 percent.

Fourth-quarter non-GAAP operating expenses were $141.9 million. This included non-GAAP R&D expense of $15.3 million, which was somewhat lower than expected due to the timing of some expenses that shifted from the fourth quarter to the first quarter of 2018. Non-GAAP SG&A expense was $126.5 million, primarily driven by the expansion of the KRYSTEXXA commercial organization. For the full year, non-GAAP operating expenses were $555.9 million.

Adjusted EBITDA was $102.7 million for the fourth quarter and $389.7 million for the full year.

The fourth-quarter non-GAAP income tax rate was 37.6 percent. The full-year non-GAAP tax rate was 31.8 percent, and cash taxes were approximately 4 percent, both in line with our expectations.

As a result of the enactment of the Tax Cuts and Jobs Act of 2017 in December, we recorded a one-time tax benefit of $74.9 million in the fourth quarter of 2017 to reflect a reduction in our net deferred tax liabilities. The benefit, which was treated as a non-GAAP adjustment, resulted from the reduction in the U.S. federal corporate income tax rate from 35 percent to 21 percent, partially offset by the write-off of a deferred tax asset related to our U.S. interest expense limitation carryforwards, due to lack of clarity in the tax legislation regarding the ability of companies to use those carryforwards in the future.

Non-GAAP net income and non-GAAP diluted earnings per share in the fourth quarter of 2017 were $48.4 million and $0.29, respectively. For the full year, non-GAAP net income and non-GAAP diluted earnings per share were $194.8 million and $1.18, respectively.

The weighted average shares outstanding used to calculate the fourth quarter and the full-year non-GAAP diluted EPS were 166.9 million shares and 165.7 million shares, respectively.

Non-GAAP operating cash flow was $157.9 million in the fourth quarter and $393.1 million for the full year, and we ended 2017 with a strong cash position of $751.4 million.

At December 31st, the total principal amount of our debt outstanding was $2.02 billion. Net debt was $1.27 billion, and our net debt to last-12-months adjusted EBITDA leverage ratio was 3.3 times.

Full-Year 2018 Guidance
Moving now to our outlook for 2018, this morning we issued our full-year 2018 net sales guidance range of $1.15 to $1.18 billion and our full-year 2018 adjusted EBITDA guidance range of $370 to $395 million. Specifically, our net sales assumptions by business unit are for:

- Full-year net sales percentage growth for the orphan business unit in the high single digits;
- Full-year net sales percentage growth for the rheumatology business unit in the high 30s; and
- Full-year net sales for the primary care business unit to exceed $350 million.

Regarding our orphan business unit guidance, as a reminder, we sold the marketing rights for PROCYSBI and QUINSAIR in Europe, the Middle East and Africa (EMEA) in late June of 2017. This will impact year-over-year comparisons for the first half of the year.

Our rheumatology business unit guidance assumes full-year KRYSTEXXA net sales growth of greater than 50 percent, which incorporates assumptions of accelerating year-over-year vial growth, the positive impact of our recently expanded KRYSTEXXA commercial organization and increased promotional efforts, as well as the potential impact of an assumed July 1, 2018 implementation of the 340B drug price rule.

We expect our non-GAAP gross profit ratio to range between 89 and 90 percent, in line with last year, and expect the first quarter to have the lowest gross profit ratio of the year, which follows a similar historical pattern.

Moving to operating expenses, as Tim mentioned, 2018 will be a year of investment in our key strategic initiatives, and our adjusted EBITDA range of $370 to $395 million reflects significantly higher year-over-year spending for teprotumumab and other R&D programs, as well as SG&A, primarily relating to the impact of the commercial expansion and promotional spending for KRYSTEXXA.

We anticipate non-GAAP R&D as a percent of sales to be in the mid-to-high single digits for the full year driven by the ramp up of our Phase 3 teprotumumab clinical program, our new rheumatology pipeline programs – HZN-002, HZN-003 and our PASylation work – as well as the KRYSTEXXA investigator-initiated trials, RECIPE and TRIPLE. We anticipate R&D spend in the first and second quarters to be the highest on a dollar basis, and in the high-single-digits as a percentage of sales. This is driven primarily by teprotumumab commercial product manufacturing and process validation work, which will occur in the first half of the year, as well as by the shift of some spend from fourth-quarter 2017 to first-quarter 2018.

We anticipate a significant increase in non-GAAP SG&A spending year-over-year, primarily reflecting the full-year impact of the expanded KRYSTEXXA commercial organization and additional KRYSTEXXA promotion-related investments. It is critical for us to invest now in KRYSTEXXA to deliver on our greater than 50 percent year-over-year net sales growth projection in 2018 and our peak annual sales estimate of greater than $750 million. And similar to prior years, we anticipate the first quarter to have the highest level of SG&A spend as a percentage of sales of any quarter during the year.

We expect full-year non-GAAP net interest expense to range between $105 and $110 million, similar to last year. This assumes that the benefit from our debt refinancing in October 2017 is offset by current LIBOR rates.

We expect a full-year non-GAAP tax rate in the high single digits, which incorporates a positive impact from the recently enacted Tax Cut and Jobs Act. As we see every year, we anticipate some variability in our non-GAAP tax rate on a quarterly basis, which averages out to the full-year estimate. We anticipate significantly higher non-GAAP tax rates in the first half of 2018 versus the second half.

We estimate that our cash tax rate will be in the mid-teens in 2018 with that rate declining to the high single digits over the next five years. As we have stated previously, this tax-rate projection could change significantly as a result of any acquisitions or divestitures made by the company.

We expect our full-year 2017 weighted average diluted share count to be between 165 and 170 million shares.

Regarding cash flow, we expect our full-year 2018 non-GAAP operating cash flow to be less than 2017. During 2017, we realized a one-time working capital benefit as a result of the implementation of the PBM contracting model for primary care. While we had a full year of rebates to the two major PBMs reflected in our net sales in 2017, we only paid eleven months of invoices for one PBM and three quarters of invoices for the other major PBM. For 2018, we expect to have a full year of rebates reflected in both our net sales and in our operating cash flows. We therefore expect this to result in a difficult year-over-year operating cash flow comparison for both the first quarter and full year of 2018.

And finally, let me cover our guidance for the first quarter of 2018. As we discuss every year, and as we see across the industry, first-quarter net sales are impacted by seasonality, as patient deductibles reset or patients experience a change in their health insurance providers. As a result, we expect first-quarter net sales to be approximately 20 percent of our full-year 2018 net sales. This is in line with prior years, when the first-quarter net sales contribution has ranged between 15 and 21 percent of full-year net sales.

We expect first-quarter adjusted EBITDA to be approximately 10 percent of our full-year 2018 adjusted EBITDA. As I mentioned in my earlier comments, we anticipate R&D to be higher in the first half of the year due mainly to timing of teprotumumab spending, and SG&A spending to be the highest in the first quarter. Our first-quarter adjusted EBITDA expectation is in line with prior years when the first-quarter adjusted EBITDA contribution has ranged between 9 and 15 percent of full-year adjusted EBITDA.

With that, I'll turn the call over to Shao-Lee.

Shao-Lee Lin, M.D., Ph.D.,
Executive Vice President, Head of Research and Development and Chief Scientific Officer

Thanks, Paul, and good morning, everyone. It's a pleasure to be joining the call this morning. I'm excited to be part of Horizon Pharma and the leadership team here as we enter the next phase of transformation in building a robust pipeline to drive sustainable growth for the long term. For me, as a physician, this commitment means greater opportunity to develop new medicines for patients with unmet needs – and in the case of rare diseases, some of the most significantly underserved patients.

Over the past two months, I've been impressed by the people here; they take their mission personally. It's a shared passion and great pleasure working with them in continuing to build our R&D capabilities and portfolio.

For today's call I'd like to highlight the programs related to our two key growth drivers, teprotumumab and KRYSTEXXA, as well as the recently added early stage-programs as next-generation opportunities to sustain leadership in uncontrolled gout.

Teprotumumab
I'll start with teprotumumab, which is a fully human monoclonal antibody that blocks the insulin-like growth factor 1 receptor, or IGF-1 Receptor. Teprotumumab is in Phase 3 development for the treatment of thyroid eye disease, or TED. If approved, teprotumumab will be the only FDA-approved medicine available to treat this rare thyroid eye disease.

In TED, IGF-1 Receptor is overexpressed on orbital tissues, the tissues surrounding the eye, and therefore, by blocking IGF-1 Receptor, it is thought that teprotumumab could block the specific autoimmune pathway involved in active TED and thereby diminish local inflammation, prevent orbital fibroblast proliferation, reduce tissue expansion, and thus restore the orbital tissue to a more normal state.

The Phase 2 clinical study results that were published in *The New England Journal of Medicine* last May were impressive, with response rate on the order of 70 percent. Significant improvements were demonstrated in proptosis, clinical activity score and quality of life. In addition, improvement in the objective, measurable endpoint of proptosis suggests potential for teprotumumab as a disease-modifying treatment.

Late last year, we initiated enrollment in our Phase 3 trial, and we continue to expect enrollment of the planned 76 patients to be completed by year-end. Titled OPTIC, this is a randomized, placebo-controlled, confirmatory trial evaluating the efficacy and safety of teprotumumab in subjects with active TED. The primary endpoint is the effect of teprotumumab versus placebo on the proptosis responder rate at Week 24, defined, as it was in Phase 2, as the percentage of patients with a reduction in proptosis of 2 millimeters or more.

At the end of the Phase 3 double-blind treatment period at Week 24, participants who are proptosis non-responders will also be eligible to enter OPTIC X, a 48-week open-label extension study in which participants may receive up to 8 additional infusions of teprotumumab. In addition, participants who are considered responders at Week 24 but who meet criteria for re-treatment due to relapse during the follow-up period off of study drug may enroll in the 48-week open-label extension, as well.

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.

KRYSTEXXA

Moving to KRYSTEXXA and our rheumatology pipeline – we saw progress across multiple fronts in the last several months.

A key component of the clinical strategy for our uncontrolled gout franchise is to improve the response rate and reduce infusion reactions to KRYSTEXXA, thereby increasing the duration of treatment possible for those patients who may develop antidrug antibodies to the medicine.

As with many biologic medicines, some people develop antibodies as part of an immune response to their medicine and therefore lose clinical efficacy with the therapy. About half of patients who take KRYSTEXXA develop this type of immune response, while the other half achieve complete response to therapy, meaning a clinically significant reduction in serum uric acid levels coupled with resolution of tophi (or uric acid deposits), tender and swollen joints, pain, and improvement in the patient's own global assessment of wellness.

Relative to degrees of response achieved with biologics across the other types of arthritis, the magnitude of complete response achieved with KRYSTEXXA is impressive. Nonetheless, we aim to improve it, as well as continue to improve understanding of the safety and proper use of KRYSTEXXA.

One of the ways we're doing this is through the support of investigator-initiated trials to examine the effectiveness of concomitant immunomodulator therapy on the rate of immune response.

One is the TRIPLE trial, an exploratory, open-label adaptive trial with multiple patient cohorts evaluating various approaches to improving the response rate of KRYSTEXXA and reducing infusion reactions. Initial data from that study was presented at the 2017 ACR meeting by the trial's lead investigator, Dr. Peter Lipsky. The data show a dramatic reduction in infusion reactions when stopping rules are followed (down to less than 1 percent from the 26 percent currently reported in the KRYSTEXXA label).

Stopping rules entail stopping treatment after two consecutive measurements of serum uric acid levels above 6 mg/dL, which can signal that the body is developing an immune response to the medicine. While stopping rules have been a frequent "real-world" practice, this was the first prospective trial to demonstrate the importance of stopping rules – and reinforces our belief in the safety of KRYSTEXXA when administered appropriately. Based on this new data, as well as additional analyses of the Phase 3 clinical trials and post-marketing safety studies, in late September of last year, we submitted a proposed update to the prescribing information for KRYSTEXXA to the FDA.

A new cohort of the TRIPLE trial will evaluate the impact to immune response of adding the immunomodulator azathioprine to KRYSTEXXA. And a second immunomodulation study, RECIPE, also an investigator-initiated trial led by Dr. Ken Saag at the University of Alabama Birmingham, will evaluate the impact to immune response with the addition of mycophenolate mofetil to KRYSTEXXA. Both azathioprine and mycophenolate are agents well known and commonly used by rheumatologists.

On the development front, we added two early-stage programs, both utilizing technologies to design new agents for uncontrolled gout with improved clinical response rates – or decreased immunogenicity – as well as the potential for subcutaneous dosing and hence improved patient convenience.

HZN-003, is a pre-clinical, genetically engineered and optimized uricase along with optimized PEGylation technology. We licensed HZN-003 in December.

The second program is a collaboration to identify clinical-stage product candidates that could use PASylation technology, adding a peptide sequence to uricase. This is hypothesized to lower immunogenicity potential relative to PEGylation and also allow potential for subcutaneous dosing.

Together, these rheumatology pipeline programs represent significant steps for the future of our uncontrolled gout portfolio, and we are excited about the prospects they offer for improved response rate and convenience of dosing in patients.

In conclusion, 2017 was a year of significant progress on the R&D front. This progress serves to catalyze the next phase of growth for Horizon Pharma and demonstrates our commitment to build a robust pipeline for sustainable long-term growth. I am looking forward to driving the continued build-out of our pipeline and R&D capabilities, so that we can deliver on our mission to bring new medicines to the patients who need them.

With that I'll turn it over to Tim for his concluding remarks.

Tim Walbert
Chairman, President and Chief Executive Officer

Thanks, Shao-Lee.

2017 was a year of significant progress for the Company:

- With the acquisition of teprotumumab, we launched the next stage of our strategy – building a pipeline of clinically meaningful medicines to drive long-term growth and shareholder value.
- We doubled the commercial organization of KRYSTEXXA to maximize its significant growth potential, which includes expanding our reach to nephrologists.
- We also delivered continued strong growth of our rare disease medicines, which underscores the value of our diversification strategy over the last several years.

In 2018, we expect continued strong performance from our rare disease medicines, which we expect to drive the double-digit year-over-year growth rate at the midpoint of our net sales guidance range. We are also investing in the long-term growth of KRYSTEXXA – and advancing teprotumumab and our growing pipeline – which will generate strong and growing long-term returns for Horizon Pharma and our shareholders.

With that, we can now open the call for questions.