Horizon Pharma plc
Third-Quarter 2018 Conference Call
Nov. 7, 2018

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Brian. 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;
- **Shao-Lee Lin, M.D., Ph.D.**, Executive Vice President, Head of Research and Development and Chief Scientific Officer;
- **Bob Carey**, Executive Vice President, Chief Business Officer; and
- **Vikram Karnani,** Executive Vice President, Chief Commercial Officer

Tim will provide a high-level review of the third quarter and an update on the business, and Paul will provide additional detail on our financial performance and guidance. Shao-Lee will discuss the clinical development programs for our rare disease medicines. After closing remarks from Tim, 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 Dec. 31, 2017, subsequent quarterly reports on Form 10-Q and our earnings press 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 press release and other filings from today that are available on our investor website at www.horizonpharma.com.

I will now turn the call over to Tim.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Tina, and good morning, everyone.

We delivered another quarter of strong performance, exceeding expectations and increasing our full-year adjusted EBITDA guidance to $420 million to $430 million. Our record quarterly net sales were driven by 25 percent growth in our orphan and rheumatology segment, including 64 percent growth in KRYSTEXXA®. This segment now represents approximately 70 percent of our business. In addition, exciting new data recently presented on our key growth drivers – KRYSTEXXA and teprotumumab – reinforce our high confidence in their ability to potentially improve the lives of uncontrolled gout and thyroid eye disease patients for many years to come.

Let me highlight the significant developments this quarter:

- With KRYSTEXXA, at the American College of Rheumatology annual meeting last month, a new nine-patient external case series showed that adding the immunomodulator, methotrexate, to KRYSTEXXA markedly improved patient response, with all nine patients responding. Based on these encouraging new findings, we are adapting our Horizon-sponsored clinical trial, the MIRROR trial, to support its potential for registration.

  We estimate peak annual net sales of more than $750 million for KRYSTEXXA, which does not assume any potential upside from our immunomodulation strategy, which has the potential to increase the number of patients responding to KRYSTEXXA as well as the average number of vials used for each patient.

- With teprotumumab, our fully human monoclonal antibody IGF-IR inhibitor in Phase 3 development for thyroid eye disease, new Phase 2 data was presented recently that reinforce our conviction that this medicine has the potential to be a disease-modifying therapy. These data also showed a durable response almost a full year off treatment for two key endpoints: proptosis, or eye bulging, and diplopia, or double vision. We presented the data at three major medical meetings that reached endocrinologists, ophthalmologists and oculoplastic surgeons – all important decision-makers in the treatment of thyroid eye disease.

Moving to our third-quarter results, we had record net sales for the orphan and rheumatology segment of $220 million, driven by KRYSTEXXA, RAVICTI® and PROCYSBI®.

KRYSTEXXA net sales for the quarter of $70 million increased 64 percent, driven by continued strong year-over-year vial growth. Following our commercial expansion that went into effect early in the year, we are generating growth in two important ways – we are opening new accounts and generating growth from existing prescribers. In fact, year-to-date, we have opened more than 450 new accounts, an increase of more than 25 percent compared to where we ended last year. In addition, vial growth of our existing prescriber base is also up significantly. The investments we are making are working, and we continue to see acceleration in the business. We expect vial growth to continue to accelerate in the fourth quarter and remain highly confident in our ability to meet our expectation of more than 65 percent year-over-year net sales growth for KRYSTEXXA in 2018.

In addition to ACR, we also attended the American Society of Nephrology meeting, where our presence laid a strong foundation for future KRYSTEXXA growth in nephrology. The traffic at our booth far exceeded our expectations, and our educational symposium was standing-room only, with more than 500 nephrologists attending. We remain excited about the significant untapped opportunity with nephrology.

Our orphan medicines, PROCYSBI and RAVICTI, generated strong net sales growth in the third quarter of 23 percent and 19 percent, respectively.

Demand remains strong for both PROCYSBI and RAVICTI, driven by patient growth and improved compliance. Both medicines are benefitting from updates to their labeled indications, which have continued to increase physician confidence in the clinical profile when treating younger, treatment-naïve patients with these medicines. We continue to expect a decision from the FDA on the RAVICTI expanded indication for the birth-to-two-years patient population by the end of this year, which, if approved, would continue to reinforce the clinical benefits of this medicine. Continued conversion from older-generation therapies, as well as the addition of treatment-naïve patients, contributed to the year-over-year patient growth for both medicines.

We continue to see tremendous opportunity for our growth drivers, which is what has driven the focused investments we are making this year. We are investing in KRYSTEXXA with our expanded commercial team, and we are investing in the teprotumumab clinical and manufacturing program as well as initiatives to prepare for its potential U.S. commercial launch.

Our goal is to deliver innovative therapies to patients and to generate strong returns for Horizon Pharma and our shareholders, and we are well on our path to achieve it.

I will now turn it 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 noted.

Third-Quarter 2018 Financial Results
Record third-quarter net sales of $325.3 million were driven by continued strong growth of our orphan and rheumatology segment.

Net sales for orphan and rheumatology were $219.9 million, an increase of 25 percent, and segment operating income was $91.5 million. As we have discussed previously, we have significantly increased our investment this year in both KRYSTEXXA, to accelerate the growth of this medicine over the long term, and in the clinical development of teprotumumab. Year to date, the orphan and rheumatology segment represents approximately 70 percent of total company net sales and total segment operating income.

Net sales for the primary care segment were $105.4 million, and segment operating income was $58.0 million.

Our non-GAAP gross profit ratio was 91.2 percent of net sales.

Non-GAAP operating expenses were $147.1 million. This included non-GAAP R&D expense of $19.1 million, reflecting investment in teprotumumab, as well as in our rheumatology pipeline programs and KRYSTEXXA investigator-initiated trials. Non-GAAP SG&A expense was $128.0 million, which was somewhat lower than expected due to the timing of some expenses that shifted from the third quarter to the fourth quarter of 2018.

Adjusted EBITDA was $149.9 million for the third quarter.

Non-GAAP income tax expense for the third quarter was $12.6 million.

Non-GAAP net income and non-GAAP diluted earnings per share were $112.6 million and $0.65, respectively. The weighted average shares outstanding used to calculate third-quarter 2018 diluted EPS were 172.5 million shares.

And non-GAAP operating cash flow was $95.6 million.

Our capital structure provides us with flexibility in managing our business. As of September 30, cash and cash equivalents were $807 million. The total principal amount of our debt outstanding was $1.993 billion, and the first maturity on our borrowings is not until 2022. Net debt was $1.186 billion, and our net debt to last-12-months adjusted EBITDA leverage ratio was 2.9 times.

On October 19, we refinanced our senior secured term loans at an interest rate of LIBOR plus 3.00 percent, a 25-basis-point-reduction from the previous interest rate, with an additional 25-basis-point stepdown to LIBOR plus 2.75 percent, if our gross leverage ratio is at or below 3.5 times. In fact, since 2016, we have lowered the interest rate on our senior secured term loans by approximately 125 basis points, saving the Company approximately $10 million in interest expense on an annualized basis. We will continue to manage our debt and leverage efficiently as we have in the past.

Full-Year 2018 Guidance

Moving now to our outlook for 2018, we expect full-year 2018 net sales to be in a range of $1.17 billion to $1.2 billion and full-year 2018 adjusted EBITDA in the range of $420 million to $430 million, an increase from our prior-guidance range of $400 million to $420 million.

We continue to expect full-year net sales growth for our orphan and rheumatology segment to be more than 20 percent. This projection includes our expectation for full-year KRYSTEXXA net sales growth of more than 65 percent and continued strong growth from our key orphan medicines, RAVICTI and PROCYSBI.

For the primary care segment, we continue to expect full-year net sales to exceed $350 million.

Regarding our guidance for other line items:

Our non-GAAP gross profit ratio is projected to range between 89 and 90 percent.

For operating expenses, we expect non-GAAP R&D as a percentage of sales to be in the mid-to-high single digits for the full year, driven by our Phase 3 teprotumumab clinical program and related work, as well as by our rheumatology development programs.

Based on timing of R&D projects this year, as well as the acceleration of our teprotumumab clinical and regulatory timeline, we anticipate our R&D spend to be meaningfully higher in the fourth quarter this year as compared to the third quarter.

We continue to anticipate a year-over-year increase in non-GAAP SG&A spending, primarily reflecting the full-year impact of our KRYSTEXXA investment, as well as initial commercial investment spend to prepare for the potential launch of teprotumumab.

We are also expecting SG&A expense to be higher in the fourth quarter due to the timing of some expenses that shifted from the third quarter to the fourth quarter of 2018 and as we increase spend in preparation for the potential launch of teprotumumab.

Full-year non-GAAP net interest expense, which is net of interest income, is expected to be somewhat lower than previous guidance at a range of $100 to $105 million.

We are projecting our full-year non-GAAP tax rate to be in the high single digits to low double digits. As we have stated previously, our tax-rate projections could change as a result of any acquisitions or divestitures.

And finally, we expect our full-year 2018 weighted average diluted share count to be between 168 and 172 million shares.

I'll now 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

Thank you, Paul, and good morning everyone.

It's an exciting time for Horizon Pharma, and particularly the R&D organization, as we grow our capabilities and advance our programs both to maximize the benefits of our existing marketed medicines as well as to build a clinically differentiated pipeline. And we've made good progress during the third quarter.

Teprotumumab
I'll begin today's update with teprotumumab – our fully human monoclonal antibody IGF-I Receptor inhibitor in development for the treatment of thyroid eye disease, or TED.

TED is one of more than 7,000 rare diseases that exist today where less than 5 percent have an approved therapy. And TED is one of the many with no approved therapy. The treatments that are nonetheless tried though not approved do not address the underlying pathogenic mechanism of the disease. We believe, based on the data demonstrated to date with the Phase 2 study, that teprotumumab has the potential to be the first approved therapy for TED, and is the first to demonstrate disease-modifying potential.

In patients with TED, IGF-1 Receptor is overexpressed on orbital tissues, resulting in local inflammation, orbital fibroblast proliferation and tissue expansion. This in turn can lead to proptosis, or bulging of the eye, and diplopia, or double vision. Patients may experience discomfort simply closing or blinking their eyes, which leads to poor sleep and can result in painful ulcers on the surface of the eye itself. Overall, the morbidity that patients experience from TED can be highly detrimental to activities of daily living, such as the ability to drive a car or even walk down stairs. And in some instances, pressure from proptosis on the optic nerve can result in blindness.

Teprotumumab's Phase 2 trial demonstrated dramatic results, which were published in *The New England Journal of Medicine* in May of 2017. After the 24-week treatment period, patients were followed for another 48 weeks – almost a full year off study drug, allowing for evaluation of durability of response post treatment.

We were pleased to present this new 72-week data for proptosis and diplopia at three medical meetings in October – the American Thyroid Association, or ATA, the American Society of Ophthalmic Plastic and Reconstructive Surgery and the American Academy of Ophthalmology.

The data presented at ATA indicated that teprotumumab has a durable proptosis response. At Week 24, the end of the treatment period, 71 percent of patients demonstrated a 2mm or more reduction in proptosis. At Week 72, approximately one year off treatment, 53 percent of teprotumumab patients that responded at Week 24 maintained at least 2mm of proptosis reduction. These results support our belief that teprotumumab offers patients a potentially disease-modifying medicine.

Data for diplopia was presented at two other conferences in October and also demonstrated a durable response almost a full year off therapy. At the end of the Week 24 treatment period, 62 percent of teprotumumab patients responded with improvement of at least one grade in diplopia, which is deemed a clinically meaningful change. At Week 72, approximately a year off treatment, 69 percent of the Week 24 responders maintained their response.

We are currently conducting a Phase 3 confirmatory trial for teprotumumab in TED entitled OPTIC. We completed enrollment in early September and expect topline results in the second quarter of 2019. In addition, we are also conducting an open-label extension study, OPTIC-X, that will allow up to an additional 24 weeks of teprotumumab treatment. Data from OPTIC-X will help inform us as to whether non-responders from the initial 24 weeks of treatment during OPTIC would benefit from longer treatment, and if patients who lose response off of drug after the initial 24 weeks of treatment would benefit from retreatment.

KRYSTEXXA and Rheumatology Programs

Moving now to rheumatology and KRYSTEXXA … a core component of our clinical strategy for KRYSTEXXA is to maximize its benefit for patients, given that it is the only FDA-approved treatment for uncontrolled gout. In the KRYSTEXXA pivotal trials, 42 percent of patients achieved complete response, maintaining a serum uric acid level of less than six mg/dL over six months. While this is impressive relative to the response rate of biologics used for other types of inflammatory arthritis, we are investigating ways to increase the number of patients who can achieve a complete response with KRYSTEXXA.

We are currently evaluating the effectiveness of concomitant immunomodulator therapy on the response rate to KRYSTEXXA, with methotrexate being the most promising and most commonly used immunomodulatory by rheumatologists like myself. There is well-documented evidence that the addition of immunomodulators to biological therapies can decrease rates of immunogenicity, as the immunomodulators work to reduce the formation of anti-drug antibodies. Three immunomodulator studies are underway – MIRROR, our Company-sponsored trial, and TRIPLE and RECIPE, two investigator-initiated studies we are supporting. Each trial is evaluating a different immunomodulator, all of which are commonly used by rheumatologists.

MIRROR is evaluating the effect on the response rate of KRYSTEXXA with the administration of methotrexate. As Tim referenced, we were very encouraged by a case series presented at ACR last month by two external investigators. The goal of the study was to evaluate the administration of methotrexate with KRYSTEXXA to improve the durability of KRYSTEXXA response. The investigators treated nine sequential patients with uncontrolled gout at three separate infusion centers. And as of October 1, six patients completed a full six-month course of KRYSTEXXA treatment, with three patients continuing to receive additional infusions. Of the nine patients followed, all were responders as defined by more than 80 percent of serum uric acid levels maintained at a goal of less than 6.0 mg/dL during the observation period.

Following these promising results, we are adapting our MIRROR trial to support the potential for registration. We had initially intended to review data from the first 15 patients in MIRROR through six months and then evaluate adapting the trial for registration. Given the external validation from the case series presented at ACR, we are moving forward earlier than planned to adapt the study and schedule a meeting with the FDA to discuss the potential for registration. In parallel, we will continue to enroll and follow patients in MIRROR. We anticipate initiation of the adapted study protocol in the second quarter of next year.

In addition, we are continuing to progress our next generation of pre-clinical programs for uncontrolled gout with the aim to support and sustain the Company's market leadership for years to come.

I look forward to future updates with you on our continued progress. With that, I'll turn it over now to Tim for his concluding remarks.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Shao-Lee.

The third quarter was another quarter of significant advancements: record quarterly net sales; record adjusted EBITDA performance resulting in our guidance raise; and new data on our two growth drivers KRYSTEXXA and teprotumumab that reinforce our confidence in their long-term peak sales potential.

In summary:

- We delivered record company net sales, driven by 25 percent growth in our orphan and rheumatology segment, which now makes up approximately 70 percent of our net sales and total segment operating income;

- KRYSTEXXA continued to deliver impressive performance, accelerating toward its full-year net sales growth target of more than 65 percent;

- We continue to advance our R&D programs. Following new data presented at ACR on KRYSTEXXA and methotrexate, we are now adapting our MIRROR clinical trial to support the potential for registration;

- We presented new data for teprotumumab that supports its disease-modifying potential and durability in the clinically important measures of proptosis and diplopia. Additionally, rapid enrollment of the Phase 3 confirmatory trial OPTIC will enable results in the second quarter of 2019; and

- We increased our adjusted EBITDA guidance range, while we continue to significantly invest in our key growth drivers, KRYSTEXXA and teprotumumab.

All of this progress is aimed at delivering innovative therapies to patients and generating sustainable long-term growth for Horizon Pharma and our shareholders. We'll now open the call for questions.

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Ashley. That concludes our call this morning. A replay of this call and webcast will be available in approximately 2 hours. Thank you for joining us.