Horizon Pharma plc
Second-Quarter 2018 Conference Call
Aug. 8, 2018

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Glenda. 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 second-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. And 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.

Today we reported strong second-quarter results and increased our adjusted EBITDA guidance range. Our strategic growth segment, orphan and rheumatology, delivered impressive performance, and we are making good progress toward our goal to become a leading rare disease medicines company with a robust development pipeline. The strong performance and momentum we are generating in the business are the result of the execution of our strategy over the last three years to rapidly diversify our business and focus on rare and rheumatic diseases. Let me provide a few highlights for the quarter:

- Our orphan and rheumatology segment achieved record net sales of $202 million. This increased 20 percent year-over-year, excluding second-quarter 2017 net sales in EMEA of PROCYSBI® and QUINSAIR™, which we divested in June last year. The orphan and rheumatology segment represents two thirds of our total Company net sales.

- Our biologic for uncontrolled gout, KRYSTEXXA®, was the Company's key growth driver. Net sales increased 53 percent year over year, driven by nearly 60 percent vial growth. The second-quarter year-over-year net sales and vial growth rates accelerated compared to first-quarter 2018, which is a direct result of our increased investment. We remain highly confident in our ability to meet our expectation of more than 65 percent year-over-year growth in 2018.

- In R&D, we have continued to make progress on several fronts:
    - Our teprotumumab Phase 3 trial has reached its target enrollment of 76 patients, well ahead of schedule. Given the new timeline, we are accelerating pre-launch activities and associated commercial investments.

    - We announced today that we will present 72-week data from the Phase 2 teprotumumab trial at the American Thyroid Association meeting in October. This will be the first time data is presented on the durability of response of teprotumumab after discontinuation of treatment.

    - And we are planning on initiating a new Company-sponsored immunomodulation study with KRYSTEXXA to continue exploring a broader clinical profile of the medicine. Titled MIRROR, this trial will evaluate if the KRYSTEXXA patient response rate is improved with the addition of methotrexate to KRYSTEXXA therapy. Along with the two ongoing investigator-initiated trials, RECIPE and TRIPLE, with the addition of MIRROR, there will be three studies underway to assess three of the most commonly used immunomodulators. Shao-Lee will discuss these developments in more detail in her remarks.

- Finally, beginning this quarter, we have implemented a new operating structure that aligns our business into two segments. This allows us to improve our operating and resource allocation decisions with our long-term strategic goals in mind.

This new operating structure is the next step in the strategic direction we set in motion several years ago to rapidly diversify the Company and become a leader in rare diseases. As we have discussed, last year we began the next phase of our strategy, which is to build a pipeline of clinically differentiated medicines. Teprotumumab is our flagship pipeline candidate, and we are actively looking for additional assets for our pipeline. Our expanded R&D leadership team, with their depth and breadth of scientific and clinical expertise, is partnering with our business development team to evaluate development-stage opportunities.

Moving to our second-quarter results, this morning we reported net sales of approximately $303 million and adjusted EBITDA of approximately $117 million. We continue to expect full-year 2018 net sales to range between $1.170 billion and $1.200 billion. We have increased our adjusted EBITDA guidance to range between $400 million and $420 million versus the prior range of $390 million to $415 million.

Growth in our orphan and rheumatology segment was driven by KRYSTEXXA, RAVICTI® and PROCYSBI.

KRYSTEXXA net sales of approximately $59 million increased 53 percent, driven by continued strong year-over-year vial growth. Our most recent commercial expansion, which went into effect the beginning of this year, has doubled our team and increased our promotional efforts to further penetrate rheumatology and initiate marketing to nephrology. Both therapeutic areas are key to our growth and success and collectively represent 100,000 patients with uncontrolled gout. The investments we are making are working, and we continue to see accelerating momentum in the business. This is evidenced by the 30 percent sequential increase in KRYSTEXXA vials sold from the first quarter.

In nephrology, we continue to see progress, with benefits investigations increasing more than 40 percent versus the first quarter. Unlike rheumatology, where we have been executing a re-education effort, nephrology is an untapped market where we see significant long-term opportunity.

Gout is a systemic disease, where, in addition to joints, it affects the organs, including the kidney. Fifty percent of the clinical trial patients in the KRYSTEXXA Phase 3 programs had chronic kidney disease. Unlike other gout medicines, KRYSTEXXA's mechanism of action is highly efficient in excreting uric acid from the kidney and can be used in patients with chronic kidney disease. These messages are resonating well with nephrologists.

We expect vial growth to continue to accelerate in the second half of this year, driven by increased demand from both rheumatology and nephrology, and this strong growth is on track to meet our guidance for full-year KRYSTEXXA net sales growth of more than 65 percent.

Our other growth drivers RAVICTI and PROCYSBI generated net sales growth in the second quarter of 21 percent and 15 percent, respectively. This excludes the impact of second-quarter 2017 EMEA net sales of PROCYSBI, which we divested in June of 2017.

Both RAVICTI and PROCYSBI are seeing continued year-over-year growth in average shipping patients. They 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. 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 near-term growth drivers, which is what has driven the focused investments we are making this year. We are investing in KRYSTEXXA to support our expanded commercial team, accelerate growth and find ways to make the medicine available to more patients. We are accelerating our investment in teprotumumab – to complete the Phase 3 clinical trial program and prepare for its potential U.S. commercial launch. And we are investing in our pipeline to drive its expansion. We have proven that our investments are working – exemplified by our transformation of KRYSTEXXA's net sales growth in two short years.

Our goal is to generate strong and growing long-term returns for Horizon Pharma and our shareholders, and we are on the 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.

Second-Quarter 2018 Financial Results
Effective this quarter, we are reporting net sales and operating income for our two segments. Corporate costs are allocated to each segment. In our earnings release, we have provided net sales and operating income for each segment for the first and second quarters of 2018 and each quarter of 2017, and the information is also posted to our website.

Second-quarter net sales of $302.8 million were driven by continued strong growth of orphan and rheumatology. This segment's net sales of $201.7 million represented 66.6 percent of our total company net sales and increased 17.2 percent year over year, driven by KRYSTEXXA, RAVICTI and PROCYSBI, and 20.4 percent after excluding the 2017 EMEA net sales of PROCYSBI and QUINSAIR.

Operating income for orphan and rheumatology was $70.6 million, representing an operating margin of 35 percent, in line with our expectations. As we have discussed previously, we are investing significantly in KRYSTEXXA this year to accelerate the growth of the medicine. We would anticipate accretion to the margin profile of this segment over time as our investments drive higher net sales.

Net sales for the primary care segment were $101.1 million. Operating income was $45.9 million, representing an operating margin of 45 percent, also in line with our expectations.

Through the first half of this year, the orphan and rheumatology segment represents approximately 75 percent of total operating income. This is tracking in line with our expectation that orphan and rheumatology should represent approximately two-thirds of our total operating income this year.

Returning to our total Company results, our non-GAAP gross profit ratio was 90.2 percent of net sales.

Total non-GAAP operating expenses were $156.6 million. This included non-GAAP R&D expense of $20.2 million, reflecting investment in teprotumumab, as well as in our rheumatology pipeline programs and investigator-initiated trials. Non-GAAP SG&A expense was $136.4 million, primarily driven by commercial investments in KRYSTEXXA.

Adjusted EBITDA was $116.8 million for the second quarter.

Non-GAAP income tax expense for the second quarter was $11.0 million.

Non-GAAP net income and non-GAAP diluted earnings per share in the second quarter of 2018 were $80.5 million and 0.48 cents, respectively.

The weighted average shares outstanding used to calculate second-quarter 2018 non-GAAP diluted EPS were 169.4 million shares.

Non-GAAP operating cash flow was $75.2 million.

As of June 30th, cash and cash equivalents were $710.2 million, net of a $23.5 million-dollar debt repayment made in June. The total principal amount of our debt outstanding was $1.993 billion. Net debt was $1.283 billion, and our net debt to last-12-months adjusted EBITDA leverage ratio was 3.6 times. Using the midpoint of our full-year guidance range, the ratio would be 3.1 times.

Full-Year 2018 Guidance

Moving now to our outlook for 2018, we expect full-year 2018 net sales to be in a range of $1.170 billion to $1.200 billion and full-year 2018 adjusted EBITDA in the range of $400 million to $420 million.

We continue to expect full-year net sales growth for orphan and rheumatology 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.

With regards to 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, which now also include our new immunomodulation study with methotrexate.

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 third and fourth quarters this year as compared to the second 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 the initial commercial investment spend for the launch of teprotumumab.

Based on these investments, as we look at the second half of this year, we would expect SG&A expense to be in a similar range to the first half.

Full-year non-GAAP net interest expense, which is net of interest income, is expected to be at the lower end of our $105 million to $110 million range.

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

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

I'll turn the call over now 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.

We continued to make progress this quarter in R&D, where we are working both to maximize the benefit of our existing medicines, such as KRYSTEXXA, as well as to build a clinically differentiated pipeline. This includes our late-stage biologic candidate teprotumumab and our next-generation gout programs, as well as active evaluation of opportunities to add to our pipeline. With the additions to our R&D leadership team this year, we have a strong and diverse team, and I am pleased to update you on our progress to date.

Teprotumumab
First, on teprotumumab – our fully human monoclonal antibody IGF-IR inhibitor in development for the treatment of thyroid eye disease, or TED. We are making great progress with the Phase 3 trial – and as you heard from Tim, the trial has reached its target enrollment of 76 patients. The remaining few subjects in screening will be allowed to randomize over the next several weeks.

As way of background, TED is one of more than 7,000 rare diseases that exist today – and one without an FDA-approved therapy. In patients with TED, IGF-1R 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 experience difficulty closing or blinking their eyes, which can cause sleep disruption and also lead to painful ulcerations. And in some cases, pressure from the bulging on the back of the eye and pressure on the optic nerve can result in blindness.

We are working to educate the medical community about TED and the potential role for teprotumumab in modifying this disease. To that end, we are pleased that the 72-week data from the Phase 2 trial will be presented at the American Thyroid Association, or ATA, meeting in October in Washington, D.C. ATA is one of the largest major medical meetings for thyroid clinicians, investigators and healthcare professionals and is an important audience for this disease.

To put the 72-week data into context, recall that the Phase 2 trial had a primary endpoint measured at week 24. This was of reduction in proptosis, or bulging of the eye, and reduction in the Clinical Activity Score, a composite score of pain, redness, and swelling that reflects ongoing inflammation. 69 percent of patients on teprotumumab vs 20 percent of those on placebo achieved the primary endpoint. Patients were followed after discontinuation of drug for another 48 weeks – almost a full year off of study drug – and allowing for evaluation of durability of response post treatment. This is the data that will be discussed in the oral presentation at ATA.

The ongoing Phase 3 trial, OPTIC, is a confirmatory trial of the Phase 2 study that was published in *The New England Journal of Medicine* last May. We are also conducting an open-label extension study, OPTIC-X, that will allow up to an additional 24 weeks of treatment with teprotumumab. The 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 relapse off drug after the initial 24 weeks of treatment, whether these patients may benefit from retreatment.

KRYSTEXXA

Moving now to rheumatology and KRYSTEXXA... A core component of our clinical strategy for KRYSTEXXA is to maximize its benefit for patients, as it is the only approved treatment for uncontrolled gout. Approximately half of patients who are treated with KRYSTEXXA achieve a complete response. And although this degree of complete response is impressive relative to response rates of biologics for other types of inflammatory arthritis, we are actively re-examining existing data and making a science-based, systematic evaluation to determine which approaches might further increase the number of patients who can benefit from KRYSTEXXA and achieve a complete response.

To that end, we are planning on initiating a new trial, called MIRROR, to continue exploring a broader clinical profile for this medicine. MIRROR will evaluate the impact of adding methotrexate to KRYSTEXXA to improve patient response, and we expect to begin enrollment in the trial in the fourth quarter. MIRROR complements the two ongoing investigator-initiated trials we are supporting – TRIPLE and RECIPE. Each of the three studies is evaluating a different immunomodulator – azathioprine, mycophenolate and now methotrexate, all well-known agents currently used by rheumatologists, with methotrexate being most commonly used, and the one that has the largest body of evidence for modulating the immunogenicity of other biologics. Evaluating three medicines gives us a number of options for future study.

During the quarter, we also continued to advance the understanding within the medical community of the long-term consequences of inadequate management of gout and the safety and efficacy of KRYSTEXXA. In June, we participated in the 2018 Annual European Congress of Rheumatology, or EULAR, medical meeting, where several abstracts on KRYSTEXXA and gout were presented. This included an epidemiology study that documented a 27 percent increase in emergency-department visits in the U.S. for gout patients between 2006 and 2014. The study results underscore the burden that gout places on patients and the healthcare system and reinforces our belief that there is a sizeable and growing uncontrolled gout population that is not being well managed. The KRYSTEXXA data presented at EULAR demonstrated how elevated serum-uric-acid levels may have systemic effects across multiple organ systems and the need to manage uncontrolled gout aggressively.

I look forward to sharing more information with you from upcoming medical meetings later this year.

To conclude, we continued to make progress this quarter in R&D, working to maximize the benefit of our existing medicines, such as KRYSTEXXA, as well to build a clinically differentiated pipeline with the aggressive advancement of development programs, including teprotumumab. We're very pleased with the accelerated progress of the teprotumumab Phase 3 trial and look forward to the data read-out next year. Our R&D leadership team continues to grow, bringing in complementary strengths and capabilities to the organization so that we can continue to expand our pipeline and also the benefits we offer to patients.

With that, I will turn it over now to Tim for his concluding remarks.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Shao-Lee.

The second quarter was another quarter of multiple advancements toward our goal of becoming a leading rare disease company:

- We delivered strong double-digit growth in our orphan and rheumatology segment, which makes up two thirds of our Company's net sales and generated record quarterly sales;

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

- We implemented a new operating structure in line with our strategic evolution that enables us to more efficiently focus on the strategic initiatives that are transforming us into a leading rare disease medicine company;

- Shao-Lee and her team are making progress in R&D. Target enrollment in our Phase 3 teprotumumab trial is now complete, well ahead of schedule. 72-week Phase 2 teprotumumab data is being presented at ATA in October and we are initiating a new study of KRYSTEXXA to continue to explore a broader clinical profile; and

- We increased our adjusted EBITDA guidance range, while we continue to invest in our near-term growth drivers, KRYSTEXXA and teprotumumab, as well as a number of programs in our pipeline.

All of this progress is aimed at driving strong and sustainable long-term growth, as well as generating returns for Horizon Pharma and our shareholders.

We will now open the call for questions.

Tina Ventura
Senior Vice President, Investor Relations

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