Horizon Pharma Plc
First-Quarter 2018 Conference Call
May 9, 2017

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Bryan. 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 first-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 then 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 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 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 made significant advancements during the first quarter toward our goal of becoming a leading rare disease medicines company with a robust pipeline of clinically differentiated medicines.

- Our orphan and rheumatology medicines drove our total company net sales growth again this quarter, with net sales of $172.5 million, an increase of 11 percent and representing more than 75 percent of our total net sales. Excluding first-quarter 2017 net sales of PROCYSBI® and QUINSAIR® in the EMEA (Europe, Middle East and Africa), which were divested in June of last year, net sales for our orphan and rheumatology medicines increased 15 percent.

- Our biologic for uncontrolled gout, KRYSTEXXA®, delivered another quarter of impressive performance, increasing 48 percent year over year. Additionally, KRYSTEXXA also showed strong sequential growth of approximately 7 percent versus the fourth quarter;

- Enrollment in the teprotumumab Phase 3 trial is tracking ahead of our expectations, with 50 percent of patients now enrolled, putting us on track to compete enrollment by year end, if not earlier.

- In January, we transformed our R&D organization with the addition of our new head of R&D and chief scientific officer, Shao-Lee Lin. She has already made great progress building her executive R&D leadership team, including therapeutic development heads for both orphan and rheumatology. We also announced several new development programs to augment our rheumatology portfolio. Shao-Lee will address these developments in her remarks shortly.

- Finally, this morning we announced a new company operating structure that takes effect in the second quarter – realigning our business into two segments: our strategic growth business – orphan and rheumatology – and primary care. This change marks a pivotal next step in our ongoing strategic transformation to a company focused on rare disease medicines. I will walk through the rationale behind this decision and how it advances our strategic goals in a moment.

For the Company overall, this morning we reported first-quarter net sales of $223.9 million and adjusted EBITDA of $33.6 million, again driven by strong growth of our orphan and rheumatology medicines.

We also increased our full-year 2018 net sales and adjusted EBITDA guidance, which now assumes a delay in the implementation of the 340B entity drug pricing rule from July 1, 2018 to July 1, 2019. Our full-year 2018 net sales guidance is now $1.170 billion to $1.200 billion, which includes an increase in KRYSTEXXA year-over-year net sales growth to more than 65 percent. Our full-year 2018 adjusted EBITDA guidance is now $390 million to $415 million.

Our new operating structure we announced this morning is the next step in the strategic direction we set in motion several years ago. In 2014, we launched the second phase of our strategy, beginning with the acquisition of Vidara and subsequently with the acquisitions of Hyperion, Crealta and Raptor. This resulted in the rapid diversification of the Company into rare diseases, assembling a portfolio of six commercial rare disease medicines.

Last year, we began the next phase of our strategy, which is to build a pipeline of clinically differentiated medicines. Our business development efforts have shifted toward acquiring a portfolio of development-stage assets. This was first marked by the acquisition of teprotumumab last year. With Shao-Lee coming on board, the new executive additions to our R&D leadership team, and also our new rheumatology development programs, we continue to make good progress. We expect to continue our aggressive business development strategy, which is to build a portfolio of development stage medicines to drive our long term growth.

The next step in our evolution is our announcement today of the realignment of our operating structure in the second quarter to operate our strategic orphan and rheumatology segment separately from our primary care segment. Structuring the business in two segments enables us to improve operating and resource allocation decisions with our long-term strategic goals in mind.

To support the new structure, we have realigned our commercial operations under a new leadership position and promoted Vikram Karnani to executive vice president and chief commercial officer. Vikram previously led our rheumatology business unit, driving the phenomenal growth and success of KRYSTEXXA. We are extremely pleased to have him in this important new role that will be integral in moving us forward in executing the growth of our commercial business. We have done the same with R&D with the addition of Shao-Lee to strengthen our orphan and rheumatology development capabilities and continue to build our pipeline.

Now, turning to first-quarter results, our orphan and rheumatology medicines had net sales of $172.2 million in the quarter, growing double digits, driven by KRYSTEXXA, RAVICTI® and PROCYSBI.

KRYSTEXXA net sales of $46.7 million increased 48 percent, driven by continued strong year-over-year vial growth of approximately 44 percent. The expansion of the KRYSTEXXA commercial organization, which we completed in the fourth quarter of 2017, is expected to lead continued acceleration of KRYSTEXXA vial growth as we educate more and more physicians about the benefits of this medicine for uncontrolled gout. This includes our new efforts with nephrologists, and we are pleased by the initial positive response that we've seen. As I mentioned, we now expect KRYSTEXXA to generate more than 65 percent net sales growth in 2018.

RAVICTI and PROCYSBI generated net sales growth in the first quarter of 12 percent and 2 percent, respectively. Excluding the impact of first-quarter 2017 EMEA net sales of PROCYSBI, which we divested in June of 2017, PROCYSBI net sales increased 12 percent. Continued conversion from older-generation therapies, as well as the addition of treatment-naïve patients, contributed to the year-over-year new patient growth for both medicines. Overall mid-single-digit growth in average shipping patients was seen across the orphan portfolio, which was driven by high-teens year-over-year growth of active shipping patients of RAVICTI.

Both RAVICTI and PROCYSBI are benefitting from updates to their labeled indications, which includes expanded use in younger patients. This has continued to strengthen physician confidence in the clinical profile when treating younger, treatment-naïve patients with these medicines.

Finally, net sales of our primary care segment of $51.7 million were lower in the quarter due to seasonality, as well as the timing of our price action. Paul will discuss this in detail shortly.

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.

First-Quarter 2018 Financial Results
First quarter net sales of $223.9 million were driven by continued strong growth of our orphan and rheumatology medicines, which represented 77 percent of our net sales and increased 11 percent over the prior year. As we see across the industry as a result of changes in insurance plans or deductible resets, first-quarter net sales were affected by seasonality, although we saw a somewhat greater impact than expected on our primary care medicines in the first quarter.

Our non-GAAP gross profit ratio was 87.0 percent of net sales, which, as we indicated previously, is expected to be the lowest gross margin quarter of the year.

Total non-GAAP operating expenses were $161.3 million. This included non-GAAP R&D expense of $15.2 million, which was lower than expected primarily due to the to the timing of teprotumumab manufacturing and process-validation activities that shifted from the first quarter to the second quarter. Non-GAAP SG&A expense was $146.1 million, primarily driven by commercial investments in KRYSTEXXA.

Adjusted EBITDA was $33.6 million for the first quarter.

Non-GAAP income tax expense for the first quarter was $3.8 million.

Non-GAAP net income and non-GAAP diluted earnings per share in the first quarter of 2018 were $4.8 million and $0.03, respectively.

The weighted average shares outstanding used to calculate the first-quarter 2018 non-GAAP diluted EPS were 168 million shares.

As I mentioned on the fourth-quarter call, we expect our full-year 2018 non-GAAP operating cash flow to be less than 2017 due to a one-time working capital benefit during 2017 of about $90 million that was the result of the timing of implementation of managed care contracts with pharmacy benefit managers for our primary care business.

Accordingly, non-GAAP operating cash flow was negative $52.7 million in the first quarter of 2018. While a full quarter of rebates to the two major PBMs were reflected in our first quarter 2017 net sales, we did not receive or pay any invoices for the PBMs during the first quarter of 2017. In 2018, the billing cycle normalized, and we had a full quarter of rebates for all PBMs reflected in both our net sales and operating cash flows. This has resulted in a difficult year-over-year operating cash flow comparison for the first quarter. This will also be a factor in the full-year 2018 comparison as well.

As of March 31, cash and cash equivalents were $674.3 million. The total principal amount of our debt outstanding was $2.020 billion. Net debt was $1.34 billion, and our net debt to last-12-months adjusted EBITDA leverage ratio was 3.6 times. Using the midpoint of our updated full-year guidance range, the ratio would be 3.3 times.

Full-Year 2018 Guidance

Moving now to our outlook for 2018, as Tim mentioned, on May 7, the U.S. Department of Health and Human Services (HHS) proposed to delay the implementation date of the 340B entity drug pricing rule for one year until July 1, 2019. As a result, we are now assuming this delay in our net sales and adjusted EBITDA guidance for full-year 2018.

We now expect full-year 2018 net sales to be in a range of $1.170 billion to $1.200 billion versus the previous range of $1.150 billion to $1.180 billion. Full-year 2018 adjusted EBITDA is now expected to be in the range of $390 million to $415 million versus the previous range of $370 million to $395 million. And we now expect KRYSTEXXA year-over-year net sales growth of more than 65 percent.

Beginning with the second quarter, we will formally report financial results for our new operating segments, reporting net sales and operating income for each, which will enable shareholders to better assess each segment.

We expect full-year net sales growth for our strategic growth aegment, orphan and rheumatology, to be more than 20 percent, which includes our revised 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. Finally, as we noted, the sale in late June 2017 of the marketing rights for PROCYSBI and QUINSAIR in Europe, the Middle East and Africa has a negative impact on the growth comparisons of those medicines in the first half of the year.

We continue to expect full-year net sales for the primary care segment to exceed $350 million. Important to understanding the quarterly run rate to achieve this full-year expectation is the fact that first-quarter primary care net sales included a $14 million dollar additional accrual for medicines in the wholesale and retail channels at the time of our February price increase. While the price increase is expected to benefit full-year net sales, in the period we effect a price increase, we are required to accrue for additional gross-to-net costs for medicines in the wholesale and retail channel. The additional accrual related to last year's price increase occurred at the end of December 2016 and, as a result, did not impact the 2017 first-quarter results. Excluding this $14 million-dollar additional accrual, primary care net sales on a pro-forma basis were roughly consistent with first-quarter 2017 results.

We expect our non-GAAP gross profit ratio to range between 89 and 90 percent, in line with 2017.

Moving to operating expenses, we continue to 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 the ramp-up of our Phase 3 teprotumumab clinical program and related manufacturing and process validation work, as well as our rheumatology pipeline programs and investigator-initiated trials. We expect R&D to increase on a dollar basis sequentially from the first quarter to the second related to the timing of some spend, including some teprotumumab CMC (Chemistry, Manufacturing and Controls) expenses that are now expected to be incurred in the second quarter versus the first.

We continue to anticipate a year-over-year 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.

Full-year non-GAAP net interest expense, which is net of interest income, is expected to range between $105 million and $110 million, as previously guided.

Our full-year non-GAAP tax rate is expected to be in the high single digits. As we have stated previously, our tax-rate projections could change significantly 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 165 million and 170 million shares.

And briefly, regarding our second-quarter expectations, we expect net sales to be 23 to 24 percent of our full-year net sales guidance and adjusted EBITDA to be 22 to 23 percent of our full-year adjusted EBITDA guidance. This is in line with our expectations for stronger performance in the second half of the year for our orphan and rheumatology medicines, particularly KRYSTEXXA, as well as for our primary care medicines.

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.

Tim has emphasized Horizon Pharma's ongoing strategic transformation into a biopharmaceutical company focused on rare disease medicines. Building a robust pipeline of clinically differentiated medicines and a strong R&D team is key to that strategy. Since I joined the company in January, one of my immediate goals has been to enhance our R&D capabilities, and I am pleased to announce, that we have added four key leadership roles to the R&D organization:

- First, a *head of development sciences*, who will be leading a number of development functions critical to successfully building our R&D capabilities, including clinical pharmacology, statistics, toxicology and biomarkers, to name a few.

- We've also added a *head of external research and development,* who will lead our R&D efforts in identifying, evaluating and executing transactions to bring in external innovation in partnership with commercial, business development and other key functions. This role will be critical to Horizon Pharma's continued growth through the licensing and acquisition of new portfolio opportunities.

- We have two new *therapeutic area heads, one for orphan and one for rheumatology,* who will each lead their respective therapeutic areas from a clinical development strategy and portfolio management perspective. These therapeutic area leadership roles also support our new operational structure and the Company's increasing focus on our strategic growth medicines.

I have known these new leaders for many years and am very pleased that they have chosen to join Horizon Pharma at this exciting time. Their proven track records, acumen and cumulative experience – which includes extensive research and development in orphan medicines, rheumatology, immunology and biopharma – will go far in helping us to quickly bolster our R&D capabilities and build out our pipeline.

And, as we build our organization and our pipeline, we constantly evaluate ongoing work to ensure it remains aligned with our strategic focus. To that end, investigator-initiated trials for ACTIMMUNE in oncology will continue to be supported with drug supply, but not additional financing, allowing us to focus on our longer-term strategic pipeline programs.

Teprotumumab
The area of focus that's most exciting to me is teprotumumab, and today we announced that we are already 50 percent enrolled in the Phase 3 trial, and therefore on track to complete enrollment by year end, if not earlier.

Teprotumumab is a fully human monoclonal antibody that blocks the insulin-like growth factor 1 receptor, or IGF-1R, to treat thyroid eye disease, or TED. As a reminder, TED appears in about 30 percent of patients with Graves' disease.

In patients with active TED, IGF-1R is overexpressed on orbital tissues, resulting in local inflammation, orbital fibroblast proliferation and orbital tissue expansion, which can lead to proptosis, or bulging of the eye.

Teprotumumab demonstrated dramatic Phase 2 results where it significantly reduced proptosis, as published in *The New England Journal of Medicine* last May. Dr. Terry Smith, one of the authors of *The New England Journal of Medicine* article, presented the Phase 2 data at ENDO, the annual meeting of the Endocrine Society, held in Chicago in March. Interest in the presentation was high. And looking forward, we plan to present additional data from the Phase 2 study from week 72, which was 48 weeks after the end of the treatment period and hence off study drug for that duration. These data will be submitted to a medical meeting later this year, and will include evaluation of the potential of teprotumumab to modify disease based on assessment of measures, including proptosis response and improvement to diplopia, or double vision, measured at the end of the treatment period, or at week 24, and at the week 72 follow-up, almost a full a year after the last dose of teprotumumab.

If approved, teprotumumab would be the only FDA-approved medicine available to treat TED.

Rheumatology Therapeutic Area

Moving now to rheumatology and KRYSTEXXA … a key component of our clinical approach for uncontrolled gout is to evaluate options to improve response rates to KRYSTEXXA. About half of patients who take KRYSTEXXA achieve complete response to therapy. 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 way we're doing this is through the support of two investigator-initiated trials, TRIPLE and RECIPE, to examine the effectiveness of concomitant immunomodulator therapy on the rate of immune response to KRYSTEXXA. The addition of immunomodulators to biological therapies has been well documented to decrease rates of immunogenicity. And both TRIPLE and RECIPE add well-known immunomodulators commonly used by rheumatologists to KRYSTEXXA with the goal of dampening the immune response during a course of treatment and decreasing the production of antidrug antibodies to KRYSTEXXA. This would potentially allow for both a longer duration of treatment and an increase in overall response rate. Both trials are open and actively recruiting patients.

To complement our work to improve the response rate to KRYSTEXXA, we are working with key opinion leaders to understand how best to characterize a complete response in uncontrolled gout, and hence inform what an optimal duration of treatment might be. This includes developing a tool to frame degrees of response. Similar to the ACR 20/50/and 70 scoring for rheumatoid arthritis, which is familiar to many, this response scale for uncontrolled gout should better help frame understanding of the magnitude of clinical impact we are achieving with KRYSTEXXA.

Another scientific area of focus for us is to advance the understanding of gout as a chronic systemic disease. Increasing evidence points to a complex relationship between high uric acid, inflammation and a variety of serious medical conditions – among them high blood pressure, chronic kidney disease and even potential for early death due to heart disease. Uric acid deposits occur not only in the joints, but also in other locations throughout the body. And data presented at ACR last year showed that decreasing serum uric acid with KRYSTEXXA has been associated with additional potentially beneficial effects, such as lowering blood pressure.

We will continue to present more data from work across these themes during the year, including upcoming medical meetings in rheumatology as well as nephrology.

We also remain excited about our two early-stage programs as next-generation approaches to uncontrolled gout that could take us further in our efforts to decrease immunogenicity, improve response rate and offer potential for subcutaneous dosing. HZN-003 is a pre-clinical program with an optimized uricase and optimized PEGylation technology. The other program is exploring PASylated uricase technology, which offers a new amino-acid based approach to extending the half-life of uricase. These programs offer additional opportunities to enhance our leadership in uncontrolled gout.

To conclude, I'll reiterate that I am very pleased with the progress we are making in the R&D organization, our new R&D leadership and our key programs. We remain committed to supporting the company's strategy to build a strong pipeline, maximize our growth medicines and enhance our R&D capabilities – with the goal to support sustainable growth and to deliver on Horizon Pharma's mission to bring new medicines to the patients who need them. I look forward to updating you on our continued progress again next quarter.

And 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 first quarter was another quarter of great progress toward our goal of becoming a leading rare disease company:

- We saw strong double-digit growth in our strategic business – our orphan and rheumatology medicines – which makes up the majority of our Company's net sales;

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

- Our Phase 3 teprotumumab trial is 50 percent enrolled and on track to complete by year end, if not earlier;

- We greatly enhanced our R&D organization – with Shao-Lee Lin joining as well as her new leadership team and the addition of several new rheumatology development programs;

- We raised our full-year guidance for both net sales and adjusted EBITDA, as well as our expectations for full-year KRYSTEXXA net sales growth;

- And, we took the next step in our evolution, announcing the new operating structure with our strategic business – the orphan and rheumatology segment – and the primary care segment – which will enable us to more efficiently focus on the strategic initiatives that are transforming us into a leading rare disease medicine company.

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.

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

Tina Ventura
Senior Vice President, Investor Relations

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