Horizon Pharma Plc
Second-Quarter 2017 Conference Call
August 7, 2017

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Nicole. 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;
- **Jeff Sherman**, Executive Vice President, Research & Development and Chief Medical Officer;
- **Dave Happel**, Executive 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 second-quarter and an update on the business. Paul will provide additional detail on our financial performance and guidance, and Jeff will provide a brief update on our clinical development programs for our rare disease medicines. 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, 2016; subsequent quarterly reports on Form 10-Q; and our earnings news release, which was issued this morning.

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

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

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

I will now turn the call over to Tim. Tim?

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Tina, and good morning everyone.

This morning we reported second-quarter net sales of $289.5 million and adjusted EBITDA of $127.0 million – both above our expectations. This was driven by significant growth of KRYSTEXXA® and RAVICTI®, as well as the strong execution within our Primary Care business. As a result, we are increasing both our full-year net sales and adjusted EBITDA guidance. Our increased net sales guidance is now $1.010 billion to $1.045 billion, up from the previous range of $985 million to $1.020 billion. Our increased adjusted EBITDA guidance is now $340 million to $375 million, up from the previous range of $315 million to $350 million. Paul will cover our financial performance and guidance in greater detail.

Second-quarter net sales for our rare disease medicines increased 70 percent. Our strategy is to accelerate organic growth of our marketed medicines and continue our disciplined business development approach, while expanding our focus to include building a rare-disease portfolio of development stage medicines.

Q1 2017 Highlights

During the quarter, we made substantial progress in executing this strategy. We initiated the expansion of our KRYSTEXXA commercial organization, where we expect to nearly double the group by year end. Our significant progress since relaunching KRYSTEXXA a year ago has given us confidence to increase our expectations for peak annual net sales to more than $400 million. Our peak sales expectation is underscored by new data presented in June at the EULAR Congress that showed a more than 400 percent increase in gout hospitalizations since 1993 at an annual cost of $43 billion. With our expanded commercial organization, we have a significant opportunity to market this highly effective medicine to many more physicians and their refractory chronic gout patients.

We also announced in the second quarter that we divested the rights to PROCYSBI® and QUINSAIR™ in Europe, the Middle East and Africa. The reason for the divestiture of this business was the alignment of resources to higher-priority, high-return businesses. We received proceeds of $72 million.

We also accelerated our strategy to add development-stage rare disease medicines with the acquisition of teprotumumab, a late-stage biologic medicine. We anticipate beginning the confirmatory Phase 3 trial by year end. Teprotumumab is in development for thyroid eye disease, or TED, a debilitating and painful condition for which there are no approved therapies. With Fast-Track, Breakthrough Therapy and Orphan Drug designations, as well as impressive Phase 2 results published in *The New England Journal of Medicine*, teprotumumab marks an important step in our strategy of assembling a portfolio of development-stage clinical candidates. We believe peak annual net sales for teprotumumab in the United States could exceed $250 million.

Last week we expanded our board of directors with two experienced senior executives, Pascale Witz and James Shannon. Pascale was most recently head of Sanofi's global diabetes and cardiovascular businesses, and before that she ran GE Healthcare's medical diagnostics business. James is the former chief medical officer at GlaxoSmithKline, and prior to that held senior leadership roles at Novartis, including the global head of pharmaceutical development. They both bring a depth of expertise and experience that will be invaluable in guiding our continued growth and transformation.

I will now review our business unit results.

Orphan Business Unit Second-Quarter Unit Net Sales
Our Orphan business unit generated $120.4 million in net sales in the quarter, an increase of 64 percent year-over-year.

RAVICTI net sales for the quarter increased 20 percent year-over-year to $47.2 million. This was driven by continued conversion of patients from older-generation nitrogen-scavenger therapies, as well as an increase in treatment-naïve patients. Active shipping patients increased more than 25 percent in the second quarter compared to last year. This was partially driven by new commercial patients following the FDA's approval in late April of RAVICTI's expanded indication in children 2 months of age to 2 years of age and older.

We expect continued double-digit net sales growth for RAVICTI in 2017, with room for additional uptake as we identify more undiagnosed and untreated urea cycle disorder patients. In addition, we continue to expect a second-half 2017 launch of RAVICTI in Europe in partnership with SOBI (Swedish Orphan Biovitrum AB).

PROCYSBI net sales in the quarter were $36.7 million, an increase of 17 percent compared to second-quarter 2016 sales of $31.4 million under Raptor. Driving this growth was demand from patients converting from older-generation therapy as well as from treatment-naïve patients, resulting in a year-over-year increase in global active shipping patients of more than 15 percent. The differentiated profile of PROCYSBI was reinforced with new clinical data this quarter highlighting its improved side-effect profile, including a reduction of body odor and bad breath – important quality-of-life issues for patients living with nephropathic cystinosis.

As a reminder, we expect the divestiture of European rights to result in a reduction of approximately $15 million to our second-half net sales, with the vast majority of this from PROCYSBI.

Second-quarter net sales for ACTIMMUNE® were $28.8 million, an increase of 10 percent sequentially from the first quarter of 2017. This was due in part to the evolution of our commercial strategy to establish the role of ACTIMMUNE in a broader range of CGD patients, including those patients awaiting bone marrow transplants. We expect ACTIMMUNE growth this year in the low single digits, driven by year-over-year growth in the second half**.**

Jeff will review our oncology pipeline opportunities with ACTIMMUNE in more detail as well.

Rheumatology Business Unit Second-Quarter Unit Net Sales
In our Rheumatology Business Unit, which includes both KRYSTEXXA and RAYOS®, second-quarter 2017 net sales increased 56 percent to $51.7 million.

KRYSTEXXA generated net sales of $38.3 million, and its strong vial growth continued – showing year-over-year growth of more than 40 percent and sequential growth in the high teens. We expect continued strong demand for KRYSTEXXA going forward.

As background, when we acquired the medicine in early 2016, KRYSTEXXA was minimally resourced and lacked a viable clinical and commercial strategy. Physician and patient feedback from the success of being treated with KRYSTEXXA gives me great confidence in our ability to drive continued acceleration in the number of patients taking this medicine. Our goal last year was to revitalize KRYSTEXXA by optimizing the commercial strategy, educating physicians about its impressive clinical data, and driving it toward the potential we knew it had. Our projected peak annual net sales at that time were for more than $250 million. And, as we made significant progress toward this initial goal more rapidly than we expected due to the execution of our commercial organization, we announced in May that we see even greater net sales potential, and that we would significantly increase our investment in KRYSTEXXA. We now project peak annual net sales of more than $400 million. And we are on track to nearly double the commercial organization, which we expect to be complete by year end.

Orphan Business Unit Second-Quarter Unit Net Sales

Primary Care second-quarter net sales were $117.4 million, a sequential improvement from first-quarter 2017 and ahead of our expectations. This was a result of better-than-expected demand as well as an increase in average net realized price, or ANRP.

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.

Q2 2017 Financial Results
I will begin with second-quarter financial results and then move to guidance.

Net sales totaled $289.5 million, an increase of 12 percent versus the second quarter of 2016, driven by continued strong growth from the Company's Orphan and Rheumatology business units.

Our non-GAAP gross profit ratio was 90.6 percent of net sales in the second quarter.

Total non-GAAP operating expenses were $135.2 million. Non-GAAP R&D expense was $12.7 million and included clinical investments in RAVICTI, KRYSTEXXA and teprotumumab. We expect non-GAAP R&D expense to increase beginning in the third quarter due to preparation for and initiation of the teprotumumab Phase 3 trial.

Non-GAAP SG&A expenses were $122.5 million, an increase of $15.4 million versus the second quarter of 2016. This was principally due to the increased commercial investments in KRYSTEXXA and expenses related to the Raptor business we acquired in October of 2016.

Second-quarter 2017 adjusted EBITDA was $127.0 million.

The income tax rate in the second quarter of 2017 on a GAAP basis was 0.8 percent and 32.2 percent on a non-GAAP basis. The non-GAAP tax rate was lower than our projected rate for the quarter, primarily due to the tax treatment of the River Vision acquisition, which was different than anticipated at the time of our first-quarter earnings call.

Non-GAAP net income and non-GAAP diluted earnings per share in the second quarter of 2017 were $68.3 million and 41 cents, respectively.

The weighted average diluted shares outstanding used to calculate non-GAAP diluted earnings per share for the second quarter of 2017 was 165 million shares.

Q2 2017 Cash Flow and Balance Sheet
Moving now to the cash flow and balance sheet for the second quarter, our GAAP operating cash flow was $47.9 million, and non-GAAP operating cash flow was $86.4 million. At June 30th, cash and cash equivalents were $554.3 million.

The total principal amount of our debt outstanding at June 30th was $2.023 billion, and net debt was $1.469 billion. Our net debt to last-12-months adjusted EBITDA leverage ratio was 3.2 times. Based on our current guidance and cash generation expectations, we expect our net debt to adjusted EBITDA leverage ratio at year-end to remain below 4 times, assuming no additional M&A this year.

Our current capital structure results in a weighted-average cash interest rate of approximately 5.4 percent based on current LIBOR rates.

FY 2017 Guidance

Moving on now to guidance – this morning we increased guidance for both our net sales and adjusted EBITDA as a result of better-than-expected second-quarter performance. Our revised net sales guidance range is $1.010 billion to $1.045 billion. The adjusted EBITDA guidance range, which is in line with our increase to net sales guidance, is now $340 million to $375 million.

Our revised net sales guidance range incorporates the following assumptions:

- Full-year net sales percentage growth for the Orphan business unit in the mid-50s;
- Full-year net sales percentage growth for the Rheumatology business unit in the mid-30s; and
- Full-year net sales to exceed $350 million for the Primary Care business unit.

Our Orphan business unit guidance reflects the reduction of approximately $15 million in net sales resulting from the divestiture of PROCYSBI and QUINSAIR rights in EMEA.

Our Rheumatology business unit guidance assumes continued strong KRYSTEXXA vial growth, driven by increased demand for this medicine. At the same time, we are now assuming a lower KRYSTEXXA average net realized price beginning in the second half, primarily based on our assumption that the U.S. government's Health Resource and Services Administration's Final Rule on 340B drug ceiling price is implemented on October 1st as currently scheduled. Unlike our other medicines, KRYSTEXXA is infused, and a portion of our vials are sold to certain hospitals that participate in the government's 340B program. This potential impact is incorporated into our 2017 guidance and growth assumptions, and our peak annual net sales estimate of more than $400 million.

Our Primary Care business unit guidance reflects the performance through the first six months of the year as well as conservative assumptions for the rest of the year.

Our other guidance metrics remain unchanged.

To recap:

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

We expect second-half operating expenses to be modestly higher than the first half of the year as a result of higher second-half R&D expenses related to teprotumumab.

We expect full-year net interest expense to range between $105 and $110 million, based on current LIBOR rates.

We continue to expect a non-GAAP tax rate for full-year 2017 in the low 30s. The year-to-date non-GAAP tax rate of 18.2 percent results in an expected non-GAAP tax rate in the mid 40s in the second half. We continue to project our full-year 2017 cash tax rate to be in the low single digits.

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

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

Jeff Sherman
Executive Vice President, Research & Development and Chief Medical Officer

Thank you, Paul.

We have begun to expand our internal clinical development pipeline to drive Horizon Pharma's long-term growth. We are working on programs that we believe will result in compelling clinical outcomes to bring first-in-kind therapies to market, such as teprotumumab, as well as optimizing currently marketed medicines through further scientific study, such as KRYSTEXXA. Our key focus is to acquire development-stage medicines through our business development efforts.

Teprotumumab
Teprotumumab is a fully human monoclonal antibody that is in late-stage development as a treatment for moderate-to-severe thyroid eye disease, or TED. TED is a rare, debilitating and very painful autoimmune disease that occurs when the body's immune system attacks the back of the eye and causes inflammation in the eye muscles and fatty tissue behind the eye, which can cause the eyes to bulge – a condition known as proptosis. More technically stated, TED is caused by immunoglobulins that activate an increase in insulin-like growth factor 1 receptor – IGF-1R activity – which results in growth of orbital tissue. Inhibiting IGF-1R attenuates the elevated hormone activity that leads to TED.

TED can occur in patients with Graves' disease, a thyroid disorder that causes hyperthyroidism, in which the thyroid gland produces excess hormone. TED has no FDA-approved therapies and with an estimated population of approximately 10,000 moderate-to-severe TED patients in the United States alone, it represents a significant unmet need. Orphan designation for teprotumumab has been granted by the FDA for this indication.

The teprotumumab Phase 2 clinical trial was the largest ever multi-center trial completed in TED. It was a double-blind, randomized placebo-controlled trial involving 88 patients and lasting 24 weeks, with an infusion of drug administered every three weeks for a total of 8 infusions. The primary endpoint of the Phase 2 trial was the response of the study eye, defined as a reduction in the Clinical Activity Score, or CAS, of 2 points or more, and a reduction of proptosis of 2 millimeters or greater at 24 weeks.

The Phase 2 trial demonstrated significant clinical efficacy in the treatment of the disease. The study's results, which were published in *The New England Journal of Medicine* in May, showed that, for the intent-to-treat study population, 69 percent of the study patients receiving teprotumumab demonstrated a statistically significant response compared to 20 percent of the placebo group at week 24, with a p value less than or equal to 0.001. In addition, the therapeutic effects were rapid, with 43 percent of the teprotumumab patients demonstrating a response versus only 4 percent of the placebo group at week 6. Other efficacy measures were significant as well.

We remain on track to begin the confirmatory Phase 3 trial by year end. It will be similar in design to the Phase 2 trial, allowing us to leverage the key learnings from that study. Furthermore, with fast-track and breakthrough therapy designations from the FDA, we will be able to submit sections of the Biologics License Application, or BLA, on a rolling basis, as well as be considered for priority and expedited review.

KRYSTEXXA
Regarding KRYSTEXXA, our strategy is to help key opinion leaders and community practitioners better understand the efficacy and safety of this medicine. Our clinical development program is focused on addressing immunogenicity both from a safety and efficacy perspective through the investigator-initiated TRIPLE trial. This adaptive design study is progressing well, and continues to enroll patients, with more than 65 enrolled, and we hope to have more data to share at future medical meetings. Furthermore, we are looking at additional strategies to address immunogenicity, including immunomodulation.

Another way we are investing in the medicine is to expand awareness of KRYSTEXXA among rheumatologists through scientific data presentations. The burden of gout on patients and the healthcare system is significant and not fully appreciated or adequately addressed. KRYSTEXXA can – and is – helping to address this problem. New KRYSTEXXA data presented at the recent EULAR Congress in June showed, among other things, that KRYSTEXXA demonstrated significant clinical benefit in both patients with and without clinically apparent tophi, and it rapidly resolved tophi in patients considered responders by substantially lowering and maintaining serum uric acid levels.

ACTIMMUNE
With ACTIMMUNE, our interferon gamma medicine in development in oncology, preclinical research indicates that interferon gamma can potentially enhance the effect of PD-1 and PD-L1 inhibitors and improve cancer-patient outcomes.

We are investing in two investigator-initiated combination therapy trials with PD-1 inhibitors, one with the Fox Chase Cancer Center and the other with the National Cancer Institute.

The Fox Chase study is evaluating whether ACTIMMUNE enhances the effect of a PD-1 inhibitor, Opdivo, in a Phase 1 oncology dose-escalation trial. The trial continues to progress well. Preliminary data in the first three cohorts of the study appear to indicate that the combination therapy is safe and well-tolerated. Patients are currently being enrolled in the fourth cohort, and we expect to have dosing-level results by the end of the year, informing the decision for proceeding to the next phase of the study.

The National Cancer Institute-supported program plans to evaluate ACTIMMUNE in combination with Keytruda, a PD-1 inhibitor, to treat cutaneous T-cell lymphoma patients. This Phase 2 study remains on track to begin later this year.

A third cancer-combination study is also underway at the Moffitt Cancer Center. It is evaluating ACTIMMUNE with Taxol, Herceptin and Perjeta to determine the optimal dosing in certain advanced breast cancer patients. While in the very early stages, the study underscores the high level of interest on the part of several academic and clinical institutions in studying ACTIMMUNE as combination therapy in certain cancers.

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

Tim Walbert
Chairman, President and Chief Executive Officer

Thanks, Jeff.

The second quarter was encouraging for us. We delivered strong second-quarter financial results and continued to execute on our rare disease medicine growth strategy, growing rare-disease sales 70 percent; adding teprotumumab, a late-stage development program, to our pipeline; and significantly increasing our investment in KRYSTEXXA's commercial efforts to drive the upside potential we see for this important medicine. We are well positioned to continue our growth strategy, and we remain focused on driving the business forward.

At this point, we will open the call for questions.