Horizon Pharma Plc
First-Quarter 2017 Conference Call
May 8, 2017

Tina Ventura
Executive Vice President, Investor Relations

Thank you, Kaylee. 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 first-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, including our announcement today to acquire River Vision Development Corporation and its biologic candidate teprotumumab. 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 regulatory filings from today that are available on our investor Web site at www.horizonpharma.com.

We have also posted an investor presentation to our website that summarizes our first-quarter results and contains additional reconciliations of non-GAAP measures to the comparable GAAP measures. Tim will reference certain slides during his remarks today.

I will now turn the call over to Tim.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Tina, and good morning everyone.

Q1 2017 Highlights
This morning we reported first-quarter net sales of $221 million and adjusted EBITDA of $52 million. Driving the
8 percent increase in net sales was the strong performance of our Orphan and Rheumatology business units with record sales from both KRYSTEXXA® and RAVICTI®.

Our Primary Care business unit results came in significantly below expectations following the implementation of a new commercial model where we are now contracting with pharmacy benefit managers and payers to help patients obtain access to our medicines.

We are addressing this underperformance, including reducing certain costs in the Primary Care business unit to align our cost structure with the lower expected sales. And with greater visibility into the impact of this transition, we are lowering our full-year 2017 net sales guidance range. It is now $1 billion to $1.035 billion and our adjusted EBITDA guidance range is now $315 million to $350 million, which includes the added R&D spend for teprotumumab, which we announced we acquired today.

We have successfully transformed the company to one that is primarily focused on rare disease medicines, which now represent 65 percent of our sales and increased 75 percent in the quarter versus last year. We are also significantly increasing investment in one of our key growth drivers, KRYSTEXXA, and as a result, we are raising our estimate of peak annual net sales for KRYSTEXXA to more than $400 million from more than $250 million.

We also announced this morning our acquisition of River Vision, which brings us teprotumumab, a biologic medicine candidate poised to enter its confirmatory Phase 3 for thyroid eye disease, or TED, in the second half of this year. In addition, this morning we announced the board's authorization of a share repurchase program for up to 10 percent of our outstanding shares.

Primary Care Business Unit First-Quarter Unit Net Sales
I will now discuss our business unit results and begin with Primary Care... Total first-quarter net sales for our Primary Care business unit, which includes PENNSAID 2%®, DUEXIS®, VIMOVO® and MIGERGOT®, were $65.6 million.

The lower net sales results relate to implementing a new contracting model, which we elected to pursue last year in order to secure broader inclusion of our primary care medicines on formularies.

Most of the agreements became effective on January 1, 2017, and we recently learned when we received first quarter rebate invoices from these PBMs at the end of April and the beginning of May that the implementation of this model has not performed in accordance with our expectations.

While our first-quarter total prescription volumes for PENNSAID 2%, DUEXIS and VIMOVO were roughly in line with our expectations, the average net realized price, or ANRP, of these medicines came in significantly below expectations.

This lower ANRP was driven by higher patient-assistance costs and higher PBM-rebate levels than we anticipated for three key reasons, which we've summarized on Slide 9 of the deck we posted on our website:

- First, we saw lower adoption rates than we anticipated for our medicines onto certain formularies. Because our medicines were not adopted – or covered by these plans – Horizon stepped in with patient support so that patients could access our medicines, and this resulted in higher-than-expected patient-assistance costs.
- Second, most of the plans that ***have*** covered our primary care medicines in the first quarter are plans that require a higher rebate, resulting in higher-than-expected rebate costs.
- And third, in the portion of our prescriptions that flow through plans that are not contracted, we have seen an accelerated level of managed care control, which equates to more restrictive or exclusionary formularies. This industry-wide trend is impacting our Primary Care business unit to a higher degree than we anticipated.

Let me take a step back and cover this in more detail. As is shown on slide 10, by way of background, PBM clients broadly fall into two categories: those clients that follow a PBM-chosen formulary and those clients that do not, often referred to as "custom clients." With PBM-chosen formularies, they are administered by the PBM. With custom clients, the PBM works on behalf of its clients to create formularies customized for the clients. The rebate amounts paid to the PBMs for clients that follow the PBM-chosen formulary are typically much higher than rebates paid to custom clients. When we entered into the PBM contracts, we assumed a certain mix between these two types of clients. And what we saw in the first quarter was a much lower adoption by custom clients than we expected.

So, why was this mix different than our assumptions?

When we established financial guidance for full-year 2017, we made estimates of the adoption rates and mix between each type of PBM client. This took into account input from the PBMs.

While we could see prescription volumes during the quarter, we did not have full visibility into the actual client mix and the magnitude of the difference until we received detailed PBM invoices at the end of April and beginning of May.

That dynamic, plus the industry-wide acceleration in managed care control resulted in the significantly lower ANRP for our Primary Care medicines in the quarter.

While we continue to work to drive adoption of primary care medicines with custom clients, we are not expecting an improvement in the level of adoption for the remainder of 2017. Therefore, we have incorporated a higher level of patient assistance costs, a higher level of rebates and a lower level of total prescription volume for Primary Care into our revised full-year 2017 net sales forecast. This has resulted in a reduction to our net sales and adjusted EBITDA guidance for the full year of 2017. We now expect Primary Care sales of more than $300 million in 2017.

So what does this mean for the Primary Care business unit moving forward? First, we still believe the transition to the contracting model was the right one to make. As we discussed with you in 2016, we have seen the trend in managed care control significantly increase – and it has further accelerated even faster in 2017. Therefore, transitioning to a contracted business model with the PBMs and payers made – and continues to make – good sense for the long-term sustainability of this business unit.

As we have been communicating for the past two years, we see Primary Care as a source of cash flow to support the expansion, diversification and growth of our Orphan and Rheumatology business units.

Further, to continue generating cash flow contributions from our Primary Care business unit, we are reducing certain costs in Primary Care and other areas of our company. We are reinvesting a portion of the funds from those cost reductions to accelerate the growth of KRYSTEXXA and support the clinical development of teprotumumab.

Rheumatology Business Unit First-Quarter Unit Net Sales

Moving now to our Orphan and Rheumatology business units – both delivered record performance in the quarter and are the growth engines for our future. I will begin with our Rheumatology business unit, which generated net sales of $42.8 million, or an increase of 56 percent. KRYSTEXXA, our biologic medicine for refractory chronic gout, generated record net sales of $31.6 million, an increase of 96 percent year over year. This was driven by the execution of our commercial organization, which drove a strong increase in year-over-year vial growth.

Last year, we increased our investment in KRYSTEXXA with additional marketing, medical education and commercial infrastructure, including new patient access managers to focus on patient and account support for additional KRYSTEXXA treatment sites. This investment is yielding the results we were planning for, and KRYSTEXXA is seeing strong momentum as a result.

Based on the continued growth of KRYSTEXXA and the clear unmet need that exists for the 40,000 to 50,000 refractory chronic gout sufferers, we are investing significant additional resources to expand our reach to physicians and increase awareness of refractory chronic gout among both physicians and patients.

Supporting this effort is an expansion of our KRYSTEXXA commercial organization to nearly 200 employees from more than 100 currently, beginning immediately and continuing through the second half of 2017. We now expect KRYSTEXXA to generate peak annual net sales of more than $400 million, up from more than $250 million.

Orphan Business Unit First-Quarter Unit Net Sales

Next I will discuss our Orphan business unit, which is now the Company's largest business unit in terms of net sales.

Our Orphan business unit generated $113 million in net sales in the quarter, up 70 percent year-over-year. Driving this strong performance was record net sales of RAVICTI, which generated net sales of $43.9 million in the quarter, an increase of 18 percent versus the first quarter of 2016, due to continued growth in active shipping patients, also up nearly 20 percent year over year.

RAVICTI has gained 43 percent market share of diagnosed patients, up from 40 percent in the fourth quarter of 2016. We expect continued double-digit growth for RAVICTI in 2017 as we identify more undiagnosed and untreated patients who can benefit from this important medicine.

PROCYSBI® also contributed to the strong performance of the Orphan business unit. PROCYSBI net sales in the quarter were $34.3 million, an increase of 25 percent compared to first-quarter 2016 sales under Raptor, of $27.5 million. An increase in active shipping patients of more than 20 percent drove these strong net sales results.

PROCYSBI is indicated for the treatment of nephropathic cystinosis, a rare and life-threatening metabolic disorder, and growth has been driven by continued strong conversion from an older-generation medicine as well as adoption of previously untreated patients. We see continued opportunity for further penetration and expect continued double-digit sales growth for the medicine in 2017.

ACTIMMUNE®'s first-quarter net sales were $26.2 million, up 3 percent. As we discussed last quarter, we have evolved our commercial strategy to establish the role of ACTIMMUNE in a broader range of CGD patients, including those patients awaiting a bone marrow transplant. ACTIMMUNE remains on track to return to growth for full-year 2017. We continue to explore additional pipeline opportunities for ACTIMMUNE with development efforts in oncology that Jeff will review in more detail.

Our Orphan business unit and KRYSTEXXA combined represented 65 percent of our first-quarter net sales compared to the roughly 40 percent in 2016 – evidence we have successfully transformed Horizon into a rare disease medicine company.

River Vision Development Corp. Acquisition

We further advanced this goal today with our announcement to acquire River Vision, an important first step in assembling a portfolio of development-stage, orphan medicines through acquisition, in-licensing and internal development.

River Vision is a privately held company with its only asset being teprotumumab, a biologic ready to enter a pivotal clinical study for thyroid eye disease, or TED. TED is a rare condition in which the eye muscles and tissue behind the eye become inflamed, causing the eyes to be pushed forward, resulting in debilitating pressure, headaches, decreased vision and in severe cases, an inability for the eye lids to cover the eyes.

With no approved medicines to treat TED, there is a significant unmet treatment need among the approximately 10,000 patients in the United States with moderate to severe disease, and we anticipate a potential peak annual sales opportunity for teprotumumab, if approved, in excess of $250 million in the United States. Importantly, teprotumumab has received Orphan Drug, Fast Track and Breakthrough Therapy designations from the U.S. FDA. It has completed Phase 2 clinical development, and the results were published last week in the *New England Journal of Medicine*. Jeff will discuss the teprotumumab clinical development program in more detail shortly.

But first, let me turn the call over to Paul to review our other financial results and guidance.

Paul Hoelscher
Executive Vice President, Chief Financial Officer

Thanks, Tim.

My comments this morning will primarily focus on our non-GAAP results.

Q1 2017 Financial Results
I'll begin with first-quarter financial results, and then move to a discussion of our full-year and second-quarter 2017 guidance.

For the first quarter, net sales totaled $220.9 million, an increase of 8 percent versus the first quarter of 2016.

Our non-GAAP gross profit ratio was 88.5 percent of net sales in the first quarter and was lower than previous quarters, primarily due to the lower ANRP in the Primary Care business unit.

Total non-GAAP operating expenses were $143.2 million. Non-GAAP R&D expense was $10.8 million and included clinical investments in KRYSTEXXA, PROCYSBI, RAVICTI and ACTIMMUNE oncology.

Beginning with the first quarter this year, we are combining sales and marketing expenses and general and administrative expenses into a single expense line item – SG&A – in order to simplify our presentation and conform to the standard practices of our peer group. For reference, we have provided two years of history in the earnings press release.

Non-GAAP SG&A expenses were $132.3 million, an increase of $27.8 million versus the first quarter of 2016. This increase was principally due to increased investment in KRYSTEXXA, which began in the second quarter of 2016, and SG&A related to the Raptor business that we acquired in 2016.

The income tax rate in the first quarter of 2017 on a GAAP basis was 34.4 percent and on a non-GAAP basis was negative 37 percent.

Non-GAAP net income and non-GAAP diluted earnings per share in the first quarter of 2017 were $35 million and 21 cents, respectively.

The weighted average diluted shares outstanding used to calculate non-GAAP diluted earnings per share for the first quarter of 2017 was 164.9 million shares.

Q1 2017 Cash Flow and Balance Sheet
And lastly, before moving to the discussion of full-year guidance, let me provide a few high-level comments about our cash flow and balance sheet.

For the first quarter of 2017, we generated $20.7 million of operating cash flow on a GAAP basis. On a non-GAAP basis, operating cash flow for the first quarter was $65.2 million.

Cash and cash equivalents were $603.4 million as of March 31st.

During the first quarter, we refinanced our senior secured term loans, consolidating the term loans at a lower interest rate and extending the maturity date from 2021 to 2024. As of March 31, the total principal amount of our debt outstanding was $2.025 billion. Please note that the refinancing had a negative impact on our first-quarter operating cash flow due to the fact that we were required to pay our term loan accrued interest balance of $5.4 million at the time of the refinancing. Our next interest payment was originally scheduled for May. This lowered our operating cash flow results for the first quarter, on both a GAAP and non-GAAP basis.

Based on the mid-point of our 2017 full-year adjusted EBITDA guidance, our net debt as of March 31, adjusted for the upfront payments for River Vision, represents a net debt to EBITDA leverage ratio of 4.7 times. Our current capital structure results in a weighted-average cash interest rate of approximately 5.3 percent, based on current LIBOR rates.

FY 2017 Guidance

Moving on to guidance … As Tim referenced, reflecting the impact of lower-than-expected first quarter Primary Care results on our expectations for the full year, our revised net sales guidance for the full year 2017 is $1.000 billion to $1.035 billion, and full-year adjusted EBITDA is $315 million to $350 million. This assumes continued strong growth for both the Orphan and Rheumatology business units, and Primary Care net sales to be more than $300 million.

Full-year non-GAAP gross margin is expected to be approximately 89 to 90 percent, a slight decrease from prior guidance due to the lower Primary Care ANRP.

Regarding our full-year operating expenses, as Tim referenced, we are reducing certain costs in our Primary Care business unit, as well as other company costs. We are investing a portion of those cost savings into KRYSTEXXA in order to further accelerate net sales growth in 2018 and beyond. Our cost-reduction initiatives, net of the KRYSTEXXA investment, will primarily affect the second half of the year. However, the benefit of the cost reduction will be offset by the investment of approximately $20 million in development-related expense associated with our River Vision acquisition, and that spending will occur primarily in the second half of 2017. Therefore, we anticipate that our quarterly operating expense for the remainder of the year on a dollar basis to be similar to the first-quarter operating expense.

For interest expense, we expect a range of between $105 million and $110 million for full-year 2017, based on current LIBOR rates.

Based on our revised guidance, we now expect a non-GAAP tax rate in the low 30s for the full-year 2017. This is higher than our previous guidance, principally due to a change in the forecasted mix of earnings by tax jurisdiction as a result of the lower net sales expected for Primary Care in 2017. Our full-year tax rate guidance for 2017 reflects the River Vision transaction announced this morning. As we have said before, any future acquisitions may impact our forecasted non-GAAP tax rate.

Relative to the share count, for the full year, we expect our weighted average diluted share count to be in a similar range as the first quarter.

2Q 2017 Guidance

For the second quarter, we expect net sales to be 23 to 24 percent of our full-year 2017 net sales guidance, and we expect adjusted EBITDA to be 18 to 21 percent of our full-year 2017 adjusted EBITDA guidance. This includes continued strong growth of our Rheumatology and Orphan business units, offset by the expected decline in Primary Care. And as a reminder, we expect second-quarter operating expense on a dollar basis to be in line with the first quarter.

We expect the non-GAAP tax rate in the second quarter to be approximately 55 to 60 percent, which, given the first quarter's favorable tax rate of negative 37 percent, would result in a first half non-GAAP tax rate consistent with our full year guidance of the low 30s.

I'll now conclude with a brief comment on our recent share repurchase authorization of up to 16 million shares. Our current intention is to execute a portion of the buyback authorization this year, depending on market conditions and our other investment opportunities.

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.

Clinical Development Programs
I will provide a brief update on our clinical development programs, concluding with a review of the teprotumumab program, which we are acquiring with the River Vision transaction.

RAVICTI®
Let us start with RAVICTI, which is indicated for UCDs. As we announced last week, we received FDA approval for a supplemental New Drug Application, or sNDA, for RAVICTI to expand the age range from patients two years of age and older to patients two months of age and older. This is an important step in helping young children with UCDs and the devastating effects of hyperammonemic events. We are also studying patients from birth to two months of age and remain on track to submit a sNDA to expand to this age range by the first quarter of 2018.

ACTIMMUNE®
Now let me touch on ACTIMMUNE and our development efforts in oncology. As we have previously discussed, we are evaluating ACTIMMUNE to enhance the effect of a PD-1 inhibitor, Opdivo, in a Phase 1 oncology dose-escalation trial with the Fox Chase Cancer Center. The trial continues to progress well.

Preclinical research indicates that interferon gamma could potentially enhance the effect of PD-1 and PD-L1 inhibitors, potentially improving cancer-patient outcomes.

As we discussed last quarter, data was presented from the Fox Chase trial at the American Society of Clinical Oncology-Society for Immunotherapy of Cancer meeting. While early, preliminary data showed that combination therapy with ACTIMMUNE and Opdivo was safe and well-tolerated in the first two cohorts, and the study continues to assess additional dose cohorts.

While the results are early, we are encouraged. The information being analyzed will inform the decision to proceed into the next phase of the study, and we look forward to additional data being made available in the coming months.

In addition to Fox Chase, a number of academic and clinical institutions have expressed interest in studying ACTIMMUNE as combination therapy in certain cancers. This includes the recent decision by the National Cancer Institute, to initiate a study later this year to treat patients with Cutaneous T-Cell Lymphoma with ACTIMMUNE and Keytruda, a PD-1 inhibitor.

KRYSTEXXA®
With KRYSTEXXA, beginning with the American College of Rheumatology meeting last November, we had a significant clinical presence where we presented KRYSTEXXA clinical data and expanded the awareness of KRYSTEXXA as an important option for patients suffering from refractory chronic gout. We are continuing to build on these efforts and look forward to presenting additional data at upcoming rheumatology meetings.

And as we have discussed before, the investigator-initiated TRIPLE trial continues to enroll patients and provide informative data. The trial is evaluating immunogenicity as it relates to KRYSTEXXA and studying a number of different subsets of patients, including those with an increased body weight.

Teprotumumab

Lastly, let me share some insight on the acquisition we announced this morning of River Vision and its late-stage-biologic candidate, teprotumumab for TED.

Teprotumumab is a fully human monoclonal antibody that is in late-stage development as a treatment for moderate-to-severe TED. It targets the Insulin-like Growth Factor-1 receptor, or IGF-1R. TED is a rare, debilitating and very painful condition that today has no FDA-approved therapy to treat it. It is associated with Graves' disease, a common thyroid disorder that causes hyperthyroidism where the thyroid gland produces excess hormone.

The treatment approach for TED today includes high-dose steroids, biologics such as rituximab, radiation and surgery. These treatments have limited efficacy, as well as safety concerns.

Because teprotumumab inhibits IGF-1R, it is in development to specifically target the underlying cause of TED where we have seen significant clinical efficacy in reduction of proptosis, which is a measure of eye protrusion and the main symptom of TED.

Teprotumumab has completed Phase 2 clinical development. The multicenter, double-blind, randomized placebo-controlled Phase 2 study lasted 24 weeks and involved 88 patients. It was the largest ever multi-center trial in TED. The study showed that 69 percent of the study patients receiving infusions of teprotumumab once every three weeks in treating active moderate-to-severe TED demonstrated statistically significant reduced proptosis and increased quality of life compared to 20 percent in the placebo group. Additionally, teprotumumab was well tolerated with hyperglycemia as the only drug-related adverse event in diabetic patients which was controlled by adjusting their diabetes medications. In fact, last week the Phase 2 clinical trial results were published in the *New England Journal of Medicine*.

The goal of the pivotal Phase 3 trial, which we anticipate beginning in the second half of this year, is to confirm the Phase 2 results. The Phase 3 trial will be similar in design to the Phase 2 trial and is currently under discussion with the FDA.

Teprotumumab has received Orphan Drug, Fast-Track and Breakthrough Therapy designations from the FDA. Fast-track designation means that we will have the ability to submit sections of the BLA dossier on a rolling basis, as well as be considered for priority review at the time of the BLA submission. Breakthrough Therapy Designation means that FDA will expedite the development and BLA review of teprotumumab. We also plan to explore other applications of teprotumumab where inhibition of IGF-1R could yield therapeutic benefits.

I look forward to sharing more with you about our clinical development programs as they advance.

With that, I will turn the call back over to Tim.

Tim Walbert
Chairman, President and Chief Executive Officer

Thanks, Jeff.

Concluding Remarks
To summarize, we delivered strong first-quarter performance in our Orphan and Rheumatology business units with record sales of KRYSTEXXA and RAVICTI, which supports our transformation to a company predominantly focused on rare disease medicines. The acquisition of River Vision and teprotumumab announced today builds on that strategy by adding a late-stage development program in our Orphan business unit.

Our cash flows and balance sheet give us flexibility to consider strategic opportunities that support our growth strategy and as well as share repurchase authorization.

Each of these initiatives allows us to generate long-term value for our shareholders.

Finally, while our Primary Care results were significantly lower than expected, we are managing the transition to a contracting model, which we continue to believe was the right decision to enhance the durability and sustainability of the Primary Care business unit over the long term.

Ultimately, our diversification strategy over the last two-and-a-half years has allowed us to shift the mix of our business from 100 percent Primary Care sales in the first half of 2014 to approximately 65 percent rare disease medicine sales in the first quarter of 2017. We believe we are well-positioned to continue our diversification strategy and drive the business forward.

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