

Horizon Therapeutics plc
Second-Quarter 2019 Conference Call
August 7, 2019

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Demetrias. Good morning everyone, and thank you for joining us.

On the call with me today are:

- **Tim Walbert**, Chairman, President and Chief Executive Officer;
- **Shao-Lee Lin, M.D., Ph.D.**, Executive Vice President, Head of Research and Development and Chief Scientific Officer;
- **Paul Hoelscher**, Executive Vice President, Chief Financial 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, after which Shao-Lee will discuss our R&D programs. Paul will discuss our financial performance and guidance, followed by 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, 2018, 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.horizontherapeutics.com.

I will now turn the call over to Tim.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Tina, and good morning everyone.

We delivered another outstanding quarter, with net sales and adjusted EBITDA both up 6 percent year over year, outperforming expectations. Importantly, net sales of our orphan and rheumatology segment grew 11 percent, driven by 36 percent net sales growth of KRYSTEXXA®. As a result, we are again raising our full-year net sales and adjusted EBITDA guidance.

Our focus on execution drove our strong second-quarter financial performance and several key milestones:

- first, our teprotumumab BLA submission in early July, an important step toward potentially bringing the first approved therapy to thousands of patients living with active thyroid eye disease (TED);
- second, the initiation of our KRYSTEXXA MIRROR trial in June evaluating if adding methotrexate to KRYSTEXXA can increase the number of patients with uncontrolled gout who benefit from this medicine; and
- third, continued improvements to our capital structure to bring it in line with that of our rare disease biopharma peers.

In addition to these milestones, we were very pleased to have Sue Mahony join our Board last week. With more than 30 years of industry experience including an 18-year tenure at Eli Lilly, Sue will significantly strengthen the Board's capabilities. Her personal investment in the lives of patients aligns directly with our mission.

I will now recap our second-quarter results and provide an update on our teprotumumab launch preparations.

In the second quarter, we generated net sales of \$321 million and adjusted EBITDA of \$124 million. Our orphan and rheumatology segment, which is our strategic growth segment that makes up 70 percent of our total net sales, drove our strong performance this quarter, increasing 11 percent year-over-year.

Demand for our orphan medicines remains strong, driven by both improved compliance as well as patient growth. ACTIMMUNE® net sales increased 7 percent year over year. PROCYSBI® patients increased 8 percent year over year, and RAVICTI® patients increased 6 percent. Similar to last quarter, RAVICTI net sales were impacted by a few factors Paul will discuss shortly.

RAYOS® was another strong performer in the quarter, with net sales growth of 51 percent, driven by continued strong execution by the RAYOS team.

KRYSTEXXA was the major driver of our second-quarter performance. Net sales increased 36 percent as a result of our commercial team's ability to drive continued growth in both existing and new accounts – including uptake in nephrology. Vial growth remains strong, up approximately 30 percent through the first half of this year. With the continued momentum from KRYSTEXXA, we now expect net sales to increase more than 20 percent for the full year.

And we still see significant untapped opportunity for KRYSTEXXA. For example, there is a great deal of headroom in our existing accounts as more and more physicians understand the full potential of KRYSTEXXA.

We continue to expect KRYSTEXXA to generate annual peak U.S. net sales of more than \$750 million. This underscores how much our commercial team has accomplished in three years since acquiring the medicine. We took an underperforming, undervalued asset and quadrupled its net sales, and we just reported 36 percent net sales growth this quarter. Not many nine-year-old medicines can claim that type of growth, and we have transformed KRYSTEXXA to the point that Horizon is now one of the most recognized leaders in gout.

KRYSTEXXA is a great example of how we optimize the value of our medicines through commercial execution – one of our hallmark strengths. We use a three-fold approach:

- first, we deeply understand the medicine, the disease and the market dynamics;
- second, we build the right team and the required infrastructure to drive uptake, support our patients and take advantage of further opportunities; and
- third, we educate physicians through a highly experienced clinical team, investing in clinical data that is important to them and improves their understanding of the disease and its treatment.

Executing on our commercial strategy has helped bring KRYSTEXXA to thousands more patients in need, which in turn has transformed its growth trajectory.

We are employing this successful approach in preparing for the U.S. launch of teprotumumab. The TED market is similar to other rare diseases in that it needs to be developed from the ground up. Today, without an approved medicine for patients, there is no defined successful treatment path. The promise of teprotumumab is that, for the first time, patients with TED would have access to a groundbreaking alternative, allowing them to circumvent years of disease progression, multiple intrusive surgeries, vision impairment and even blindness. Given the dramatic efficacy of teprotumumab, we expect the current paradigm to shift – and that shift will require a significant amount of disease education and market development.

We have made a great deal of progress on both fronts since our update last quarter. Our sales force is hired. They have completed training, and they are out in the field building relationships with the treating physician community, which includes oculoplastic surgeons, ophthalmologists and endocrinologists. In addition to our approximately 50-person sales force, we have hired our patient educators, reimbursement specialists and a dedicated site-of-care team. In total, this roughly 100-person team will support teprotumumab and the TED community. Together they bring a tremendous level of talent to Horizon, with broad collective experience and deep relationships within the physician community, as well as experience with infused medicines.

Our ongoing market development and access activities include:

- educating the treating physicians on active TED, and the importance and urgency of treatment;
- strengthening the co-management of the disease across key potential prescribers; and
- establishing the treatment path, infrastructure and referral network necessary for an infused therapy. This is especially important as ophthalmologists and endocrinologists rarely infuse medicines today. These physicians need to be educated on the processes involved to ensure patient access.

Our Medical Affairs team, including field medical liaisons supporting teprotumumab, is also fully in place, and we look forward to sharing Phase 3 teprotumumab data at key ophthalmology and endocrinology meetings this fall. This includes the American Thyroid Association and American Association of Ophthalmology meetings, as well as the fall meeting of the American Society of Ophthalmic Plastic and Reconstructive Surgery, or ASOPRS, where we will be presenting Phase 3 diplopia data for the first time. We are also engaging at meetings of specialty societies on the local and community level, partnering with organizations dedicated to infusion therapy and connecting with relevant patient advocacy groups. All told, we are building a strong foundation across a broad spectrum of TED stakeholders and engaging with each of them to ensure they understand the severity of TED, the patient journey and the urgency with which this disease needs to be treated.

And we continue to see a tremendous amount of interest in teprotumumab both from the physician and patient communities – which is understandable, given teprotumumab’s impressive efficacy data and the fact that there is currently no approved treatment. That’s why we are excited about the possibility of teprotumumab becoming a key treatment option for patients suffering from this vision-threatening, serious and progressive disease – and also why we were pleased to recently announce the Expanded Access Program for teprotumumab, which Shao-Lee will discuss momentarily. This is an exciting time for us at Horizon and for the TED community as a whole.

I will now turn it over to Shao-Lee to provide an update on R&D.

Shao-Lee Lin, M.D., Ph.D.,
Executive Vice President, Head of Research and Development and Chief Scientific Officer

Thank you, Tim, and good morning everyone.

Teprotumumab

We continue to make excellent progress in R&D, with several key developments. I'll start with teprotumumab, which we submitted for FDA approval in early July. As part of the submission, we requested Priority Review, given that teprotumumab has Breakthrough Therapy, Orphan Drug and Fast Track designations. The FDA has a 60-day filing review period to determine if the BLA is complete and acceptable for filing. If the FDA accepts the filing and grants Priority Review, we would expect a six-month review period beginning on the filing acceptance date, with potential approval in the first quarter of 2020.

The BLA submission included results from both OPTIC, our Phase 3 trial, as well as results from the Phase 2 trial, which were published in *The New England Journal of Medicine*. OPTIC confirmed the Phase 2 results and also demonstrated a highly statistically significant effect on the primary endpoint of reduction in proptosis of 2 mm or more, with an 82.9 percent response rate in the teprotumumab treatment group versus 9.5 percent in the placebo group, and a p-value of less than 0.001. All secondary endpoints were also met, with a p-value of less than or equal to 0.001.

We presented additional Phase 3 data at an endocrinology meeting in April that included the key secondary endpoint of absolute proptosis reduction. These data demonstrated that after the full six-month course of therapy, the teprotumumab group achieved a proptosis reduction of 3.32 mm compared to 0.53 mm for placebo. We look forward to sharing additional data from the Phase 3 trial at upcoming ophthalmology and endocrinology meetings later this year.

TED is a serious, progressive and vision-threatening autoimmune disease for which there is no FDA-approved medical intervention. The disease is believed to be medically treatable only during the active stage. Once it progresses to the inactive stage, the effects are permanent, and surgery becomes the only option. Multiple surgeries per eye may be required, and even then, results are not always optimal. Patients may still experience visual impairment despite surgical intervention. For patients to wait two years – the average length of active TED – for the disease to burn out also poses serious challenges.

During its active stage, TED is painful and debilitating. Many patients can't close their eyelids; their eyes become dry and irritated, which ultimately can lead to corneal ulcerations. Today, patients are treated with steroids or other agents that are off-label and with no proven effectiveness on proptosis, which is the primary driver of morbidity in TED and can cause diplopia, or double-vision. Many feel stigmatized due to their appearance and can suffer from serious depression.

The debilitating nature of the disease and the limited timeframe for potential treatment underscore the urgency to treat, which is why we are working to make teprotumumab available as soon as possible – and which is why we were very pleased to have recently announced an expanded access program for teprotumumab. This program was developed in partnership with the FDA and is designed to provide access to teprotumumab for patients who meet protocol eligibility criteria while the BLA is under review. Such patients might otherwise experience further progression of TED prior to potential approval.

KRYSTEXXA and Uncontrolled Gout Programs

Moving now to KRYSTEXXA and our programs for uncontrolled gout...During the second quarter, we initiated our MIRROR immunomodulation trial, evaluating whether the administration of KRYSTEXXA in combination with methotrexate can increase the response rate of KRYSTEXXA, allowing more patients living with uncontrolled gout to receive a full course of treatment. The randomized, placebo-controlled trial is expected to enroll approximately 135 patients to receive either KRYSTEXXA plus methotrexate or KRYSTEXXA plus placebo. The primary endpoint is defined as the proportion of patients maintaining serum uric acid levels of less than 6 mg/dL at Month 6.

One of the reasons we are excited about the MIRROR trial is the positive results from a case series conducted by two external investigators evaluating the administration of KRYSTEXXA with methotrexate on patient response rate. The original case series data was presented at the American College of Rheumatology medical meeting last fall, with all nine sequential patients studied who had uncontrolled gout achieving positive response, which was defined as more than 80 percent of serum uric acid levels being maintained at less than 6 mg/dl during the observation period. In June, at EULAR, the European League Against Rheumatology meeting, the investigators shared updated data, including a tenth sequential patient. All 10 patients completed the course of therapy and achieved a positive response.

Gout was a topic of significant interest at EULAR. As we have previously noted, there is a growing body of evidence about the systemic impact of high serum uric acid levels and also the co-morbidities associated with gout. One presentation was particularly compelling: it demonstrated in a randomized clinical trial that profoundly lowering serum uric acid levels led to improved kidney function in patients with diabetic nephrology, suggesting that lowering serum uric acid could slow the progression of chronic kidney disease.

Given that kidney transplant patients have more than a tenfold increase in the prevalence of gout when compared to the general population, and that chronically elevated levels of serum uric acid are associated with transplant organ rejection, we will be evaluating KRYSTEXXA in kidney transplant patients with uncontrolled gout in our own study. On track to begin in the second half of this year, this study will serve to further inform nephrologists as to the use and effectiveness of KRYSTEXXA and its potential benefit for their chronic kidney disease patients with uncontrolled gout.

Finally, we are making good progress in our early-stage gout programs. Our discovery and development collaboration with HemoShear has identified a number of novel targets, and recently achieved the milestone of moving into the validation phase of those targets, ahead of schedule.

To conclude, the second quarter was another one of significant progress, and I look forward to updating you again next quarter on the continued advancement of our programs.

With that, I'll now turn the call over to Paul.

Paul Hoelscher
Executive Vice President, Chief Financial Officer

Thanks, Shao-Lee.

My comments this morning will primarily focus on our non-GAAP results, unless otherwise noted.

Second-Quarter 2019 Financial Results

Second-quarter net sales of \$320.6 million were driven by another quarter of strong commercial execution. Our orphan and rheumatology segment generated net sales of \$223.5 million in the second quarter, an increase of 11 percent, driven primarily by KRYSTEXXA, along with RAYOS, PROCYSBI and ACTIMMUNE.

Operating income for orphan and rheumatology was \$74.5 million, representing an operating margin of 33 percent, in line with our expectations. As we have previously discussed, we are investing significantly in teprotumumab and in our rheumatology pipeline programs. We anticipate accretion to the margin profile of this segment over time as our investments drive higher net sales.

Demand for RAVICTI remains strong, with year-over-year patient growth up 6 percent. Similar to the first quarter, net sales in the quarter were impacted by a lower net price related to higher Medicaid rebate accruals, which we do not anticipate in the second half of the year. RAVICTI net sales were also impacted by the divestiture of the ex-U.S. rights late last year. As a result of both factors, RAVICTI net sales decreased 11 percent.

Net sales for the inflammation segment, previously known as the primary care segment, were \$97.1 million, and segment operating income was \$49.7 million. We continue to invest cash flow generated from this segment into the orphan and rheumatology business.

Our non-GAAP gross profit ratio was 90.9 percent of net sales for the quarter.

Non-GAAP operating expenses were \$167.4 million. This included non-GAAP R&D expense of \$22.0 million, reflecting investment in teprotumumab, as well as in our rheumatology pipeline programs. Non-GAAP SG&A expense was \$145.4 million.

Adjusted EBITDA was \$124.1 million for the second quarter.

Non-GAAP income tax expense for the second quarter was \$12.2 million.

Non-GAAP net income and non-GAAP diluted earnings per share were \$95.6 million and \$0.49, respectively.

The weighted average shares outstanding used to calculate second-quarter 2019 non-GAAP diluted EPS were 193.2 million shares.

And non-GAAP operating cash flow was \$95.7 million for the quarter.

As Tim mentioned, in 2019 we began aligning our capital structure to be closer to those of our rare disease biopharma peers, which generally have lower debt levels. So far this year, we have reduced our gross debt by \$575 million, while maintaining a strong balance of cash and cash equivalents, which totaled \$866 million at June 30. Net debt was \$577 million at June 30, and our net leverage ratio, defined as net-debt-to-the-last-12-months adjusted EBITDA, was 1.1 times, down two-and-a-half turns from 3.6 times only one year ago. In July, we issued \$600 million of 5.5 percent senior notes due in 2027 and are using the proceeds, along with cash on hand, to repay \$625 million of our outstanding debt. Through our refinancing and debt reduction initiatives, we have lowered our annualized cash interest expense by more than 40 percent versus a year ago, and extended the maturity of our term loans and senior notes by two to four years.

Full-Year 2019 Guidance

Moving to our outlook for 2019, we now expect full-year 2019 net sales to range between \$1.28 billion to \$1.30 billion versus the previous range of \$1.26 billion to \$1.28 billion. We project full-year net sales growth for KRYSTEXXA to be more than 20 percent, and we continue to expect the full-year net sales for the inflammation operating segment to be roughly similar to 2018. Should there be an at-risk generic launch of VIMOVO®, we do not expect it to have a material impact on our 2019 net sales guidance.

In addition, we recently learned that PENNSAID® 2% may be added to Express Scripts' 2020 exclusion list. While the list is not final until annual negotiations are complete, if PENNSAID 2% is on the list, we expect the impact to 2020 total company net sales to be immaterial. As a reminder, PENNSAID 2% has always been on the Caremark exclusion list. We evaluate all of our PBM contracts to ensure that we can achieve patient access as well as generate a return. Our objective is to continue to generate strong cash flows from the inflammation segment to invest in orphan and rheumatology.

Returning to 2019 guidance, full-year 2019 adjusted EBITDA is now expected to be in the range of \$460 million to \$475 million versus the previous range of \$450 million to \$465 million.

We continue to project our non-GAAP gross profit ratio to be approximately 90 percent.

Non-GAAP R&D expense as a percentage of net sales is now expected to be in the mid-to-high single digits for the full year. We expect non-GAAP SG&A expense to increase meaningfully in the second half compared to the first half, as a result of the previously discussed investment in U.S. teprotumumab launch preparations.

We continue to expect a full-year non-GAAP tax rate in the low-to-mid teens and a cash tax rate in the low-to-mid single digits. In line with previous years, we anticipate quarterly variability in our non-GAAP tax rate, and, as always, this projection could change significantly as a result of any acquisitions or divestitures or any changes in tax laws.

We now expect full-year non-GAAP net interest expense to be approximately \$65 million, a decrease from our previous guidance of approximately \$70 million and from our initial guidance of \$90 million to \$95 million, due to the significant improvements that we have made to our capital structure so far this year.

We continue to expect full-year 2019 weighted average diluted share count to be approximately 190 million shares.

With that, I will turn the call over to Tim for his concluding remarks.

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Paul.

We delivered another outstanding quarter – outperforming expectations and increasing our full-year guidance. But more important than our latest quarterly results is what we have achieved for the long-term success of Horizon in an incredibly short amount of time. Through exceptional execution, we have transformed Horizon into one of the leading profitable rare disease biopharma companies, validated by several impressive successes:

- We have built a portfolio of rare and rheumatic disease medicines, growing at a double-digit pace and driving the future growth of the company.
- We successfully relaunched KRYSTEXXA, on track for more than 20 percent growth this year, nine years post approval and three years post acquisition – and we are on track to achieve our projected peak annual U.S. net sales of more than \$750 million.
- We have executed on our strategy to expand our pipeline with the successful development of teprotumumab and a potential approval in the first quarter of next year. We are preparing aggressively for the launch, leveraging our success with KRYSTEXXA to make teprotumumab as successful – if not more – and continue to project peak annual U.S. net sales of more than \$750 million if teprotumumab is approved.
- We have significantly improved our capital structure, taking advantage of market opportunities to reduce our gross debt, lower our interest expense and extend our debt maturities. With more than \$850 million in cash and a net leverage ratio of 1.1 times, our balance sheet has never been healthier. We are in a great position to build our pipeline and take advantage of additional acquisition opportunities that meet our criteria.

Our business fundamentals and financial position are strong, and we are well positioned for significant future growth – with perhaps one of the best long-term growth opportunities in our space. We remain focused on executing our strategy, which we believe will lead to long-term success and value for Horizon, for patients and for our shareholders.

With that, we'll now open it up for questions.

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Demetrias. 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.