

Horizon Therapeutics plc
First-Quarter 2019 Conference Call
May 8, 2019

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Jimmy. 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 first quarter and an update on the business and Shao-Lee will discuss the clinical development programs for our rare disease medicines. Paul will then provide detail on our financial performance and guidance. After closing remarks from Tim, we will take your questions.

As a reminder, during today's call we will be making certain forward-looking statements, including statements about financial projections, our business strategy and the expected timing and impact of future events. These statements are subject to various risks that are described in our filings made with the Securities and Exchange Commission, including our annual report on Form 10-K for the year ended Dec. 31, 2018, subsequent quarterly reports on Form 10-Q, and our earnings press release, which were 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 are off to a strong start this year, reporting first-quarter results that outperformed expectations. We achieved net sales of \$280.4 million, representing 25 percent year-over-year growth, and adjusted EBITDA of \$88.4 million, up 163 percent year over year.

As a result of our strong execution, we are raising our full-year net sales guidance range to \$1.26 billion to \$1.28 billion and our full-year adjusted EBITDA guidance range to \$450 million to \$465 million. Importantly, we are raising guidance while further increasing our investment in the potential U.S. launch of teprotumumab, our biologic in development for active thyroid eye disease, or TED.

In addition to our strong commercial performance, we are executing on our pipeline strategy. We announced unprecedented results from our teprotumumab Phase 3 trial in the first quarter, which was a major milestone for the Company. The Phase 3 results along with the Phase 2 data form a highly convincing body of clinical evidence that supports our planned mid-year U.S. regulatory submission. Preparation for the U.S. commercial launch continues; we are investing in market development and market access activities to bring teprotumumab to patients who have no other options, as well as investing in a second manufacturing site to increase commercial supply for our long-term needs. I will discuss our plans and our thoughts on the active TED market in a moment.

Regarding our uncontrolled gout pipeline programs, we have now finalized the design of our KRYSTEXXA[®] immunomodulation trial, the MIRROR trial, and expect it to begin next month. As a reminder, we have adapted this trial for registration to evaluate the effect of adding methotrexate to KRYSTEXXA to improve the patient response rate. In addition, during the quarter, we selected a lead PASylated uricase candidate, HZN-007, that we intend to advance further in development. Shao-Lee will discuss our R&D progress in more detail.

In line with our evolution to an R&D-focused company, last week shareholders approved the changing of our name to Horizon Therapeutics plc. Our new name more clearly reflects both our long-term strategy to develop and commercialize innovative new medicines to address rare diseases, as well as the fact that our work with patients, caregivers, physicians and communities goes well beyond our medicines.

I will now summarize our first-quarter results. Our orphan and rheumatology medicines generated net sales of \$185.9 million, driven by KRYSTEXXA, RAYOS[®] and PROCYSBI[®]. Net sales for this segment increased 10 percent, excluding the divested ex-U.S. rights for RAVICTI[®], BUPHENYL[®] and LODOTRA[®].

KRYSTEXXA net sales of \$52.3 million increased 12 percent, meeting our expectations, and driven by more than 30 percent year-over-year volume growth. The KRYSTEXXA team continues to execute extremely well, opening more than 600 new accounts over the last 12 months. Vial growth in existing accounts increased approximately 30 percent compared to the first quarter of last year. We continue to see strong interest from nephrologists, with good sequential growth, and are looking forward to initiating our trial in kidney transplant patients later this year. Demand remains strong as we enter into the second quarter, and we remain on track for our double-digit full-year net sales growth expectation. RAYOS generated strong double-digit growth during the quarter as well, driven by continued focused execution by this team.

Demand remained strong for both PROCYSBI and RAVICTI in the first quarter, driven by mid-single-digit year-over-year patient growth and improved compliance. PROCYSBI net sales increased 13 percent year-over-year. RAVICTI net sales increased 5 percent, excluding the impact of divested rights for RAVICTI outside of North America and Japan.

Our first-quarter outperformance has put us in a very strong position for the year – allowing us to increase our investment in teprotumumab while also increasing our full-year guidance.

I will conclude my remarks this morning with an update on our teprotumumab U.S. commercial launch preparations and our latest thoughts on the active TED market.

Following the dramatic Phase 3 results, we have received significantly more interest in teprotumumab from patients, caregivers and physicians. As I mentioned, we are increasing our investment in teprotumumab, and the pace of that investment, to successfully bring this medicine to patients as quickly as possible upon approval.

Based on a significant amount of research we have conducted internally and with external parties, we have a very good understanding of the U.S. TED patient population. As is done for most rare diseases with limited published data, we have built our estimates using patient-level data, including actual claims and hospital admissions data. As we have previously discussed, we estimate that 15,000 to 20,000 active TED patients are eligible for teprotumumab treatment each year. This is the annual incident patient population. Because TED patients have active disease for up to three years, a portion of those patients roll over into the following year and are additive to the teprotumumab eligible patient population each year.

Given the impressive efficacy data for teprotumumab, we expect significant interest and demand from both physicians and patients for an approved therapy. Patients with TED are desperate for treatment given that there is no approved treatment today.

The active TED market is one we need to build from the ground up, which is typical with rare diseases and requires significant investment in market development and market access. Today, with no well-defined treatment path or successful patient journey for those with active TED, endocrinologists and ophthalmologists in the United States typically take a “watch and wait” approach until patients progress from active TED to inactive TED. At that point, they are referred to an oculoplastic surgeon to perform surgery to reduce proptosis, or eye bulging, and its deleterious effects. The surgery is highly complex, invasive and often only be partially effective – and often needs to be repeated multiple times given the technical challenges in this disease.

If teprotumumab is approved, we believe that this treatment paradigm will shift. Early on, we see ophthalmologists and oculoplastic surgeons playing a larger role, and once the market is more established, we believe that both endocrinologists and ophthalmologists will play a central role in co-managing active TED. From the qualitative feedback we have received to date, key opinion leaders and clinicians across all specialties are very enthusiastic about this potential treatment option.

A significant amount of market development is required to identify and educate potential treating physicians and help them get their active TED patients to a place where they can receive proper administration of therapy. This requires a dedicated infrastructure that we are in the process of building out. It includes sales professionals, medical affairs professionals, patient educators and reimbursement specialists. As teprotumumab is an infused therapy, both endocrinologists and ophthalmologists, who

primarily prescribe oral medicines, will need to establish a treatment path, infrastructure and a referral pattern to ensure patient access and success.

We remain extremely encouraged by the opportunity we see for teprotumumab and are even more confident today in our annual peak U.S. net sales guidance of more than \$750 million. We are excited about the benefit teprotumumab could potentially offer to the many patients living with the challenges and pain of active TED. We look forward to updating you as our BLA (biologics license application) submission and launch activities progress.

I will now turn the call over to Shao-Lee.

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

The first quarter of 2019 marked a milestone quarter for us as we reported positive topline results for our teprotumumab Phase 3 confirmatory trial. As Tim mentioned, we achieved our primary endpoint of reduction in proptosis of 2 millimeters or greater, with an impressive 82.9 percent response rate in the teprotumumab group compared to 9.5 percent in the placebo group and with a p-value of less than 0.001. Proptosis, or eye bulging, is the main cause of morbidity in TED. All secondary endpoints were also met with a p value of less than or equal to 0.001. The safety profile was consistent with that reported in the Phase 2 trial, with no new safety events noted relative to Phase 2.

In patients with active TED, the IGF-1 Receptor is overexpressed on orbital tissues, resulting in local inflammation, orbital cellular proliferation and tissue expansion, which in turn causes proptosis. Patients may experience discomfort simply closing or blinking their eyes, which can lead to poor sleep patterns and result in painful ulcers on the surface of the eye itself. Proptosis can also result in diplopia, or double vision. Overall, the morbidity that patients experience with active TED can be highly detrimental to activities of daily living, such as the ability to drive a car, read or even walk down stairs. In some instances, pressure on the optic nerve from proptosis can even result in blindness.

Currently, no pharmacotherapy is approved for the treatment of active TED. By blocking IGF1-Receptor signaling, teprotumumab is believed to specifically target the downstream autoimmune pathophysiology that underlies active TED, resulting in the efficacy and safety seen in the clinical program.

At the AACE meeting two weeks ago, we shared additional secondary endpoint data from the Phase 3 trial. These data showed a rapid reduction in proptosis, reaching statistically significant differences at the first measured time point of Week 6. The secondary endpoint of mean proptosis reduction showed an average reduction over the span of the trial – essentially, the average of how patients did at Weeks 6, 12, 18, and 24 altogether – and demonstrated an average of 2.82 millimeters for the teprotumumab group versus 0.54 millimeters for placebo, with a p value of less than 0.001.

Perhaps more clinically meaningful is the proptosis reduction seen from baseline to Week 24, representing proptosis response after the full six-month course of therapy. That result from baseline to Week 24 was a 3.32-millimeter reduction for the teprotumumab group vs 0.53 millimeters for placebo. Additionally, the overall responder rate at Week 24, which was the primary endpoint in Phase 2 (and was defined as proptosis reduction of more than 2 millimeters plus Clinical Activity Score improvement of 2 points or more), was significantly better for patients treated with teprotumumab. The overall responder rate was 78.0 percent for patients treated with teprotumumab compared with 7.1 percent in placebo.

These Phase 3 results give us confidence that teprotumumab has the potential to become the first approved therapy for active TED, and if approved, could improve the lives of many people living with this debilitating disease. We remain on track to submit a BLA in mid-2019, with Breakthrough Therapy, Orphan Drug and Fast Track designations. We anticipate the potential for approval in the first half of 2020.

We are also evaluating an early access program for teprotumumab, given the level of interest we have received since sharing topline results from the Phase 3 confirmatory trial. And we continue to explore ways to further advance our understanding of the mechanisms through which IGF1-Receptor is providing clinical benefit, such that we can consider potential for benefit in additional patient populations. We look forward to sharing more with you on these investigations in the future.

KRYSTEXXA and Uncontrolled Gout Programs

Moving now to KRYSTEXXA and our uncontrolled gout programs, we continue to make progress towards our goal of maximizing the benefit of KRYSTEXXA for patients. As we have discussed in the past, in the KRYSTEXXA pivotal Phase 3 trials, 42 percent of patients achieved complete response, maintaining a serum uric acid level of less than six milligrams per deciliter over six months.

In the MIRROR study, we are investigating the use of the immunomodulator methotrexate to enhance the response rate of KRYSTEXXA and increase the number of patients who can benefit from it. We have finalized the trial design. It is a randomized placebo-controlled study that will enroll approximately 135 patients to receive either KRYSTEXXA plus methotrexate or KRYSTEXXA plus placebo. The primary endpoint is a comparison of the proportion of responders, defined as patients with serum uric acid levels of less than 6 milligrams per deciliter, at six months between treatment arms. We remain on track to initiate this study in June.

As discussed last quarter, in support of our strategy to expand KRYSTEXXA's use among nephrologists, we are also initiating a clinical trial in the second half of 2019 evaluating KRYSTEXXA in kidney transplant patients. Managing uncontrolled gout is both a common and significant unmet need in kidney transplant patients. This trial will also serve as an opportunity 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.

Across both the MIRROR trial and the study of KRYSTEXXA in kidney transplant patients, we are gaining additional information to advance our understanding of gout as a chronic systemic disease, such as a better understanding of the effects of KRYSTEXXA on uric acid deposition in organs beyond the joints.

Finally, we've made good progress in our early-stage, next-generation biologic programs for uncontrolled gout. We are working on two programs with different technologies to enhance response rates and target subcutaneous dosing. One program is evaluating a PASylated uricase technology, and we recently selected a lead candidate, known as HZN-007. The other program underway optimizes PEGylation as well as the uricase, and is HZN-003. We are committed to rigorous research and development in the area of uncontrolled gout to enhance our leadership position in this space and improve the therapies available for gout patients.

As always, I look forward to updating you on our continued progress, and I'll now turn the call over 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.

First-Quarter 2019 Financial Results

First-quarter net sales of \$280.4 million were driven by another quarter of strong commercial execution. Our orphan and rheumatology segment generated net sales of \$185.9 million in the quarter, an increase of 8 percent, and generated segment operating income of \$46.7 million, also an increase of 8 percent. As we have discussed previously, we are no longer recognizing RAVICTI and BUPHENYL net sales outside of North America and Japan, or any net sales of LODOTRA.

Excluding the impact of those divestitures, net sales for our orphan and rheumatology segment increased 10 percent, and RAVICTI net sales increased 5 percent. As Tim referenced, demand for RAVICTI remains strong, driven by year-over-year patient growth along with improved compliance and adherence. We also recently gained additional insight into downstream inventory levels, which along with an increased Medicaid mix, has resulted in a need to accrue somewhat higher Medicaid rebates, resulting in a lower net price for RAVICTI in 2019 compared to 2018.

Net sales for the primary care segment were \$94.5 million, and segment operating income was \$41.4 million. This exceeded our expectations, driven by continued execution by the commercial team, which has led to stabilization of this business over the last several quarters. Importantly, we are investing the cash flow from this segment into the orphan and rheumatology segment, including increasing our investment in the preparation for the potential U.S. launch of teprotumumab.

Our non-GAAP first quarter gross profit ratio was 89.8 percent of net sales.

Non-GAAP operating expenses were \$163.8 million. This included non-GAAP R&D expense of \$17.1 million, reflecting investment in teprotumumab, as well as in our rheumatology pipeline programs. Non-GAAP SG&A expense was \$146.7 million.

Adjusted EBITDA was \$88.4 million for the first quarter.

Non-GAAP income tax expense for the first quarter was \$12.8 million.

Non-GAAP net income and non-GAAP diluted earnings per share were \$53.9 million and \$0.30, respectively.

The weighted average shares outstanding used to calculate first-quarter 2019 non-GAAP diluted EPS were 180.3 million shares.

Non-GAAP operating cash flow was \$62.2 million.

We continue to execute on our capital allocation strategy, and are well on our way to achieving our goal of aligning our capital structure to be closer to that of our aspirational biopharma peers. As of March 31st, cash and cash equivalents were \$1.033 billion. On March 11th we closed an underwritten public equity offering of 14.1 million ordinary shares and we received net proceeds of \$326.8 million. We used

the net proceeds along with cash on hand to fund the repayment of \$300 million of our term loans in March and \$250 million of our senior notes due 2023 on May 1, reducing the principal amount of our debt outstanding to \$1.443 billion. As of March 31, our net debt was \$660 million, and our net leverage ratio, defined as net-debt-to-the-last-12-months adjusted EBITDA, was 1.3 times. This compares to 3.6 times at March 31, 2018 – a reduction of more than two turns compared to a year ago.

Full-Year 2019 Guidance

Moving to our outlook for 2019, we now expect full-year 2019 net sales to be in the range of \$1.26 billion to \$1.28 billion versus the previous range of \$1.23 billion to \$1.25 billion. We continue to project double-digit full-year net sales growth for KRYSTEXXA, and we now expect primary care net sales for the full year to be roughly similar to 2018.

Full-year 2019 adjusted EBITDA is now expected to be in the range of \$450 million to \$465 million versus the previous range of \$440 million to \$455 million.

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

We continue to expect non-GAAP R&D expense as a percentage of net sales to be in the high single digits for the full year. We anticipate a higher year-over-year increase in non-GAAP SG&A expense, which primarily reflects the investments we are making to prepare for the potential teprotumumab U.S. commercial launch, which we have increased following receipt of the Phase 3 data in late February. Following the repayment of \$550 million of our outstanding debt that I mentioned earlier, we expect full-year non-GAAP net interest expense to be approximately \$70 million, a decrease from our previous guidance of between \$90 and \$95 million.

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

Following our recent equity offering, we now expect our full-year 2019 weighted average diluted share count to be approximately 190 million shares.

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

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Paul.

In summary:

- We are off to a great start in 2019, exceeding expectations on both net sales and adjusted EBITDA.
- The outperformance we achieved this quarter allowed us to raise our full-year guidance while at the same time further increasing our investment in the potential U.S. commercial launch of teprotumumab.
- The first quarter of 2019 was significant from an R&D perspective, with two key milestone developments:
 - The dramatic teprotumumab Phase 3 trial results that we announced in February and the additional data we presented at AACE two weeks ago are highly significant and give us increased confidence that teprotumumab has the potential to be the first approved therapy for active TED patients and improve the lives of many people living with this debilitating disease; and secondly,
 - Our KRYSTEXXA immunomodulation trial, MIRROR, is on track to begin in June.
- Looking to the future, both of these high-growth opportunities, KRYSTEXXA and, if approved, teprotumumab, could more than double our full-year 2018 total company net sales – with each expected to generate more than \$750 million in peak net sales.
- We also completed our underwritten public offering in March, which helped us reduce our gross debt by \$550 million and reduce our net leverage ratio to 1.3 times, well below our target.
- Finally, we have changed our name to Horizon Therapeutics, which more clearly reflects both our long-term strategy to develop and commercialize innovative new medicines addressing rare diseases, as well as the fact that our work with patients, caregivers, physicians and communities goes well beyond our medicines.

With that, we would now like to open up the call for your questions.

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Jimmy. 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.