

Horizon Therapeutics plc
Fourth-Quarter 2019 Conference Call
February 26, 2020

Tina Ventura
Senior Vice President, Investor Relations

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

On the call with me today are:

- **Tim Walbert**, Chairman, President and Chief Executive Officer;
- **Liz Thompson, Ph.D.**, Group Vice President, Clinical Development and External Search;
- **Paul Hoelscher**, Executive Vice President, Chief Financial Officer;
- **Vikram Karnani**, Executive Vice President, Chief Commercial Officer; and
- **Andy Pasternak**, Executive Vice President, Chief Business Officer

Tim will review our 2019 performance and 2020 guidance. Liz will then provide a review of our R&D programs, followed by Paul, who will discuss our financial performance. After closing remarks from Tim, we will then take your questions.

As a reminder, during today's call we will be making certain forward-looking statements, including statements about financial projections, our business strategy and the expected timing and impact of future events. These statements are subject to various risks that are described in our filings made with the Securities and Exchange Commission, including our annual report on Form 10-K for the year ended Dec. 31, 2019, and our earnings press release, which we 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.

Our fourth quarter results capped off another year of tremendous progress for Horizon. I will review the highlights and then provide more detail on our performance.

- We achieved record full-year net sales of \$1.3 billion – driven by 32 percent KRYSTEXXA® growth and adjusted EBITDA of \$483 million – which included our significant investment in TEPEZZA™ launch preparations. Excluding this investment, our underlying business generated strong double-digit adjusted EBITDA growth for the year.
- TEPEZZA is our new biologic for thyroid eye disease, or TED, and was approved on January 21st, nearly two months ahead of the PDUFA date. This was followed the next day by *The New England Journal of Medicine* publication of our Phase 3 trial results. We have seen an incredible level of excitement and demand for TEPEZZA in the first month on market. I will share more shortly.
- For KRYSTEXXA, our uncontrolled gout biologic and other key growth driver, we announced topline results from our MIRROR open-label trial that demonstrated a 79 percent complete response rate when used with methotrexate. This result is roughly double the 42 percent rate seen with KRYSTEXXA in its Phase 3 program and was the third consistent data set with a complete response rate of 79 percent or better.
- Based on expectations for continued adoption of KRYSTEXXA with methotrexate, continued growth of new and existing accounts and further penetration in nephrology, we increased our KRYSTEXXA peak U.S. net sales estimate to more than \$1 billion earlier this year. We also increased the peak U.S. net sales estimate for TEPEZZA to more than \$1 billion.
- We significantly improved our capital structure in 2019, paying down \$575 million of gross debt, extending our debt maturities and reducing our annualized net interest expense by more than 40 percent. Our strong balance sheet puts us in a great position to acquire or in-license medicines to expand our development-stage pipeline.
- And this morning we issued full-year 2020 guidance of \$1.40 billion to \$1.42 billion in net sales and \$485 million to \$500 million of adjusted EBITDA. Paul will go through this in more detail.
- As we look to 2021 and beyond, we expect to generate significant operating margin expansion driven primarily by the growth of TEPEZZA and KRYSTEXXA. We believe our double-digit net sales growth and strong double-digit earnings growth targets over the 2020 to 2023 timeframe will make Horizon one of the fastest growing companies in our space.

I will now provide additional commentary on our key medicines, beginning with TEPEZZA.

TEPEZZA

Last year was certainly a landmark year for TEPEZZA. It began with the February release of our Phase 3 trial results, with the primary outcome of 83 percent of patients achieved a dramatic improvement in proptosis versus 9 percent for placebo. We submitted our BLA in July, ahead of expectations, and following a highly successful FDA Advisory Committee meeting in December, we received early FDA approval on January 21st. We are pleased to be the first company to provide thyroid eye disease patients an approved medicine to treat this rare, vision-threatening and disfiguring disease.

Our launch is off to a strong start. The patient and physician feedback we have received exemplifies our vision at Horizon to go to incredible lengths to make healthcare a priority, not just a privilege.

In fact, one TED patient was scheduled for strabismus surgery, which is a complex eye alignment surgery. However, following our first physician education program, his surgeon decided TEPEZZA would be a better option – and there are many more stories like that. And it was a momentous day for us on February 5th, when the first TEPEZZA patient started treatment – less than two weeks after approval.

This substantial interest certainly reflects the promise of TEPEZZA. It's also due to the significant market education efforts we started well before approval. Our 100-person TEPEZZA team has been in the market since last July, building key relationships and educating key stakeholders.

Our strategy for TEPEZZA is focused on four key areas:

- First, establish the market structure and simplify the patient journey, which had been long, poorly defined and very frustrating given there was no approved medicine or even standard of care;
- Second, educate all stakeholders on TED and TEPEZZA, including physicians, patients, patient advocacy organizations, infusion centers, sites of care as well as payors;
- Third, support the launch with our comprehensive, high-touch, patient-centric model; and
- Fourth, facilitate access to TEPEZZA by establishing an infusion site-of-care referral network, which is so important for this medicine.

While we are still early in our launch, about a month in, we have made excellent progress:

- Since approval, our team has called on over 75 percent of our 1,300 top-tier physician accounts.
- We now have more than 500 patient enrollment forms, or PEFs, in the queue – which has far exceeded our expectations at this early date. PEFs are similar to prescriptions or benefit investigations and an indication of the strong initial demand. Importantly, about 10 percent of the PEFs are from physicians who are not priority targets, which reflects the significant interest from the broader TED community for TEPEZZA. The vast majority of physicians submitting PEFs are oculoplastic surgeons or from ophthalmology subspecialties.
- While this PEF number indicates very strong demand, conversion remains subject to payor and reimbursement dynamics, including an up-to-60- to 90-day delay to infusion while we while we have a temporary J-code.
- Like all Part B infused medicines, we launched TEPEZZA with a temporary code. We submitted our application in early February for a permanent J-code that is expected to go into effect on October 1st. With this permanent code, we anticipate that the reimbursement approval process should become much more efficient.
- Our payor team has meetings scheduled with nearly 70 percent of our top payor targets, representing nearly 80 percent of total lives. Conversations related to access and coverage have been positive at this time.
- Our site of care team has made great progress as well. Since approval, they have activated more than 150 infusion centers, including regional and national accounts. Activated accounts are now ready to administer TEPEZZA provided the payor approves coverage.
- And finally, our direct-to-patient and direct-to-physician media campaigns have been highly successful. Given the disfiguring and vision-threatening nature of TED and the fact that two-thirds of patients are female, this is a patient population that can be activated to ask for TEPEZZA themselves. So, we're not surprised that visitors to TEPEZZA.com have been very engaged. In fact, "click-through" rates – or the percentage of people who click on our ad and then go to our site – have been running at more than 20 percent, dramatically higher than the healthcare industry standard of 3 to 5 percent, which underscores the exceptionally high interest in TEPEZZA. There has been a significant level of social media engagement from physicians, patients and even infusion centers – across Twitter,

Facebook and Instagram. We are very pleased that the community is so enthusiastic about the promise for TEPEZZA.

Based on the early success we have had to date, we continue to feel very confident in our more than \$1 billion peak U.S. net sales expectation given the unmet need in this market.

Orphan and Rheumatology

We will now briefly cover our orphan and rheumatology segment, which represented approximately 75 percent of our total net sales and increased 14 percent in the fourth quarter.

KRYSTEXXA

KRYSTEXXA was again the major driver of our performance with fourth-quarter net sales increasing 33 percent, driven by strong vial growth. We also exceeded our full-year growth target of more than 25 percent, ending the year with growth of 32 percent.

We increased our KRYSTEXXA peak U.S. net sales expectation to more than \$1 billion earlier this year and expect more than 25 percent growth again in 2020. The commercial, medical affairs and clinical teams have done a fantastic job of reinvigorating this brand – not many 9-year-old medicines can lay claim to that type of growth. Even so, only 4 percent of the 100,000 addressable patients in the United States used KRYSTEXXA in 2019, meaning there is substantial opportunity for continued growth.

We see three areas of growth for KRYSTEXXA: continuing to expand existing accounts and new accounts; increased use of KRYSTEXXA with immunomodulators such as methotrexate; and continued acceleration of growth in the nephrology space, where we are seeing increased uptake of KRYSTEXXA from the efforts of the dedicated nephrology sales force we put in place last year.

Physicians are using immunomodulators such as methotrexate, more frequently, as they continue to see evidence that it dramatically increases the KRYSTEXXA patient response rate. This includes positive data generated by two external investigators that demonstrated complete response rates of 100 percent and 80 percent when methotrexate was used with KRYSTEXXA. In January, we added to this body of evidence with the topline results from our MIRROR open-label trial that demonstrated that adding methotrexate to KRYSTEXXA resulted in a significant 79 percent complete response rate, almost doubling the rate seen in the Phase 3 clinical program. A separate investigator recently published use of KRYSTEXXA with multiple immunomodulators, including methotrexate, azathioprine and cyclosporine, and also achieved a complete response rate of 100 percent in these patients.

We have analyzed claims data to understand the growth in physician use of immunomodulators with KRYSTEXXA. In early 2019, it was running in the single digits. We recently reviewed data through October of last year – before our MIRROR open-label results were public – which showed that physician use has grown to the low double-digits. For KRYSTEXXA used with methotrexate, the response rate is significantly higher, allowing patients to potentially stay on therapy longer. This higher likelihood of patient success increases physician confidence in prescribing KRYSTEXXA to more of the patients in their care.

Moving on to our rare disease medicines, demand remains strong, driven by both improved compliance and net patient growth. Average shipping patients increased mid-single digits across ACTIMMUNE®, RAVICTI® and PROCYSBI® combined for the quarter. With the expanded indications for PROCYSBI and RAVICTI, many new patients are younger, starting treatment on lower doses, which will typically increase as they grow older. Our strategy is driven by patient identification, diagnosis and ongoing support of patients throughout their treatment journey.

I will now turn the call over to Liz who will discuss our R&D progress in more detail. Liz led the TEPEZZA Phase 3 clinical program and the TEPEZZA Advisory Committee meeting and was integrally important to our success with TEPEZZA and early approval. Liz is leading our clinical development organization. Liz?

Elizabeth Thompson., Ph.D.

Group Vice President, Clinical Development and External Search

Thank you, Tim, and good morning, everyone.

I'm pleased to be with you today to provide an update on our key pipeline programs, beginning with TEPEZZA.

TEPEZZA

The approval of TEPEZZA in January was the culmination of a tremendous amount of work. This effort spanned our organization, but it wouldn't have happened without the patients living with thyroid eye disease and the physicians who partnered with us on the clinical development program. I echo Tim's comments about how pleased we are to bring this important new medicine to thyroid eye disease patients who until now had no other options.

Shortly after approval, the TEPEZZA Phase 3 results were published in *The New England Journal of Medicine*. It's a mark of the importance of the data that the results of both TEPEZZA's Phase 2 and Phase 3 trials were published in this esteemed medical journal. We anticipate sharing additional data this year at key conferences for ophthalmologists, oculoplastic surgeons and endocrinologists.

We continue to advance OPTIC-X, the extension trial of the TEPEZZA Phase 3 trial. OPTIC-X was designed to explore whether patients who lose response off drug after the initial 24 weeks of treatment would benefit from retreatment and whether non-responders from the initial 24 weeks of treatment during the Phase 3 trial would benefit from longer treatment. We expect to report top-line results on OPTIC-X early next year.

Now that TEPEZZA is on the market for thyroid eye disease, we are evaluating other indications to maximize the value of this medicine for patients. By blocking the IGF-1 receptor, TEPEZZA may have the potential to positively impact fibrotic processes in other diseases. To further evaluate this potential, we are embarking on an exploratory trial in diffuse cutaneous scleroderma, which we anticipate initiating mid-year.

Diffuse cutaneous scleroderma, a subset of scleroderma, is a rare chronic and systemic autoimmune disease marked by fibrosis, including hardening of the skin and internal organs, potentially causing significant organ damage. There are roughly 30,000 diagnosed patients in the U.S., primarily managed by rheumatologists – but there are no approved treatments for this rare disease.

KRYSTEXXA

It was a quarter of important developments for KRYSTEXXA as well. We continued our efforts to maximize the benefit that KRYSTEXXA offers patients with uncontrolled gout through our two MIRROR immunomodulation trials. The MIRROR program was designed to evaluate whether methotrexate can help dampen the immune response to KRYSTEXXA and thereby increase the patient response rate, allowing more patients to complete a full course of therapy. We have results in hand now from the first of these, the MIRROR open-label trial, showing that 11 of 14 enrolled patients achieved a complete response, and we've submitted these data for presentation at a medical meeting later in the year.

Our 135-patient MIRROR randomized clinical trial progressed during the quarter, and we are on track to complete enrollment mid-year. Data are expected in 2021, and if positive, we would pursue an update to the KRYSTEXXA prescribing information.

We also continue to advance PROTECT, our 20-patient open-label trial evaluating KRYSTEXXA in kidney transplant patients with uncontrolled gout. Kidney transplant patients have more than a tenfold higher prevalence of gout when compared to the general population, and chronically elevated levels of serum uric acid can be associated with transplant organ rejection. We believe this trial will provide helpful data to nephrologists in treating high-need patients.

And finally, in line with our strategy to maximize the value of our medicines, we will be initiating an open-label trial with KRYSTEXXA to evaluate a significantly shorter infusion duration. Today the infusion process takes approximately 2 hours. Reducing that time would meaningfully improve convenience for patients, which we believe could also result in greater treatment compliance. We expect a mid-year start to this trial.

We are keenly focused on building our pipeline through M&A and licensing, and my team is actively working with our business development colleagues to evaluate additional medicines to add to our pipeline.

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

Paul Hoelscher
Executive Vice President, Chief Financial Officer

Thanks, Liz.

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

Fourth-Quarter 2019 Financial Results

Fourth-quarter net sales of \$363.5 million were driven by 14 percent growth of our orphan and rheumatology segment. Net sales for this segment were \$269.8 million, and operating income was \$95.4 million. This was in line with our expectations and reflects the significant investments that we have been making in the launch of TEPEZZA and our pipeline.

Net sales for the inflammation segment were \$93.7 million, and segment operating income was \$44 million. For the full-year 2019, we generated higher operating income in this segment compared to 2018, despite modestly lower net sales. Our strategy is working – we are reinvesting the cash flow generated from this segment into our key growth drivers TEPEZZA and KRYSTEXXA.

As noted in our press release this morning, beginning with the first quarter of 2020, we are changing the name of the orphan and rheumatology segment to the orphan segment and are restructuring our commercial operations to move RAYOS to the inflammation segment, as it shares similar call points with the other inflammation medicines. Therefore, beginning with the first-quarter results, RAYOS will be reflected in the inflammation segment. In addition, with its launch in the first quarter, TEPEZZA net sales will be included in the orphan segment.

Our non-GAAP fourth quarter gross profit ratio was 90 percent of net sales.

Non-GAAP operating expenses for the fourth quarter were \$187.7 million. This included non-GAAP R&D expense of \$26.5 million, reflecting investment in TEPEZZA, as well as our gout pipeline programs. Non-GAAP SG&A expense was \$161.1 million, reflecting increased launch preparation expenses for TEPEZZA.

Adjusted EBITDA was \$139.9 million for the fourth quarter, exceeding expectations.

The non-GAAP income tax rate in the fourth quarter was 9 percent, resulting in a 6.7 percent non-GAAP tax rate for the full year. On a GAAP basis, in the fourth-quarter we recorded a benefit of \$559.9 million primarily related to an intracompany transfer of intellectual property assets.

Non-GAAP net income was \$116.6 million and non-GAAP diluted earnings per share were \$0.56. The weighted average shares outstanding used to calculate fourth-quarter 2019 non-GAAP diluted EPS were 211 million shares. This higher share count incorporates the impact of the potential conversion of our \$400 million of exchangeable notes into ordinary shares. Given our recent share price appreciation, these notes are approaching the point where we can call them. Based on our current expectations, we are required to include the shares from this potential conversion in our fourth-quarter and full-year 2019 GAAP and non-GAAP diluted EPS calculations. Despite this impact, our non-GAAP diluted EPS still exceeded expectations for the fourth quarter and the full year.

Cash Flow and Balance Sheet

Non-GAAP operating cash flow was \$192.0 million in the fourth quarter and \$446.4 million for the full year.

As of December 31, cash and cash equivalents were \$1.076 billion, giving us significant flexibility to manage our business, accommodate our growing operations and expand our pipeline.

As Tim highlighted, we significantly strengthened our capital structure in 2019, achieving our objective to be more closely aligned to profitable R&D-focused biopharma peers, which generally have lower leverage levels. We ended the year in a significantly stronger position, with gross debt of \$1.418 billion and net debt of \$341.7 million. Our net-debt-to-last-12-months adjusted EBITDA leverage ratio of 0.7 times is about a third of our 2.3 times net leverage at the end of 2018. The rating agencies have recognized this, with S&P and Moody's both upgrading our corporate ratings to BB- and Ba3, respectively.

Full-Year 2020 Guidance

Moving to our outlook for 2020, this morning, we provided full-year net sales guidance of \$1.40 billion to \$1.42 billion, underscoring our expectation for another year of strong commercial execution, driven by KRYSTEXXA, TEPEZZA and our other orphan medicines.

For KRYSTEXXA, we project net sales growth of greater than 25 percent in 2020, based on expectations for continued strong demand from rheumatology and accelerating nephrology adoption.

For TEPEZZA, as we mentioned on our approval call, we expect 2020 launch-year net sales to be in the range of \$30 million to \$40 million. This assumes a more gradual net sales uptake in the first half given the additional time required for the manual approval process associated with a temporary J-code and the establishment of a referral infusion network. We expect the majority of our 2020 sales to occur in the second half of the year, as the approval process becomes more efficient after the permanent J-code takes effect on October 1st.

We expect less than \$5 million for VIMOVO net sales in our 2020 guidance, given the expected at-risk generic launch.

We expect full-year adjusted EBITDA in the range of \$485 million to \$500 million, reflecting strong net sales performance offset by our launch-year investments for TEPEZZA and additional investments in our pipeline programs.

Our non-GAAP gross profit ratio is expected to be approximately 90 percent.

We expect operating expenses to increase in 2020, primarily related to a year-over-year increase in SG&A for the launch of TEPEZZA, which has been reflected in our adjusted EBITDA guidance. We continue to expect our non-GAAP R&D expense as a percentage of sales to be in the mid-to-high single digits for 2020.

We expect full-year non-GAAP net interest expense to be approximately \$50 million as a result of our capital structure improvements in 2019.

For our income tax rate, we expect a full-year non-GAAP tax rate in the high single digits. As we see every year, we anticipate variability in our non-GAAP tax rate on a quarterly basis.

We estimate that our cash tax rate will be in the double-digits in 2020, decreasing in 2021 and beyond. As always, this projection could change significantly as a result of any acquisitions or divestitures made by the Company or any changes in tax laws.

We expect our full-year 2020 weighted average diluted share count to be in the range of 210 million to 212 million shares, similar to the fourth quarter of 2019.

And finally, let me touch on the first quarter. As we discuss every year, first quarter net sales are generally the lowest of the year, impacted by seasonality as patients experience changes in their health insurance coverage. In addition, first quarter operating expenses are also typically the highest of the year, and this year even more so, driven by higher TEPEZZA launch expenses early in the year, with the majority of TEPEZZA sales in the second half of the year.

Therefore, we expect first-quarter net sales to be somewhat more than 20 percent of our full-year 2020 net sales, in line with prior years. We expect first-quarter adjusted EBITDA to be in the low double digits as a percentage of our full-year 2020 adjusted EBITDA, also in line with prior years.

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

Tim Walbert
Chairman, President and Chief Executive Officer

Thank you, Paul.

2019 was a year of tremendous progress and performance at Horizon, marked by the achievement of several important milestones. It is also a testament to the strong execution of our strategy to maximize our growth drivers and expand our pipeline for sustainable growth.

- We generated record net sales of \$1.30 billion and adjusted EBITDA of \$483 million, despite significant investment made in the development and U.S. launch preparations for TEPEZZA.
- We also invested in several new R&D programs for KRYSTEXXA to maximize the benefit it offers patients and to enhance our leadership in uncontrolled gout, and we recently expanded our pipeline with the addition of two new programs.
- During 2019, we significantly improved our capital structure and aligned it more closely with our profitable biopharma peers, reducing our gross debt by \$575 million, extending our debt maturities and lowering our annualized net interest expense by more than 40 percent.
- Our continued strong execution has generated substantial value for shareholders, with our total shareholder return considerably exceeding the Nasdaq Biotechnology Index over the last 1, 3 and 5 years.
- We are entering 2020 in our strongest position ever as a company, with the year already off to an excellent start. We look forward to updating you on our progress throughout the year.

With that, we will open it up for questions.

Tina Ventura
Senior Vice President, Investor Relations

Thank you, Michelle. 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.