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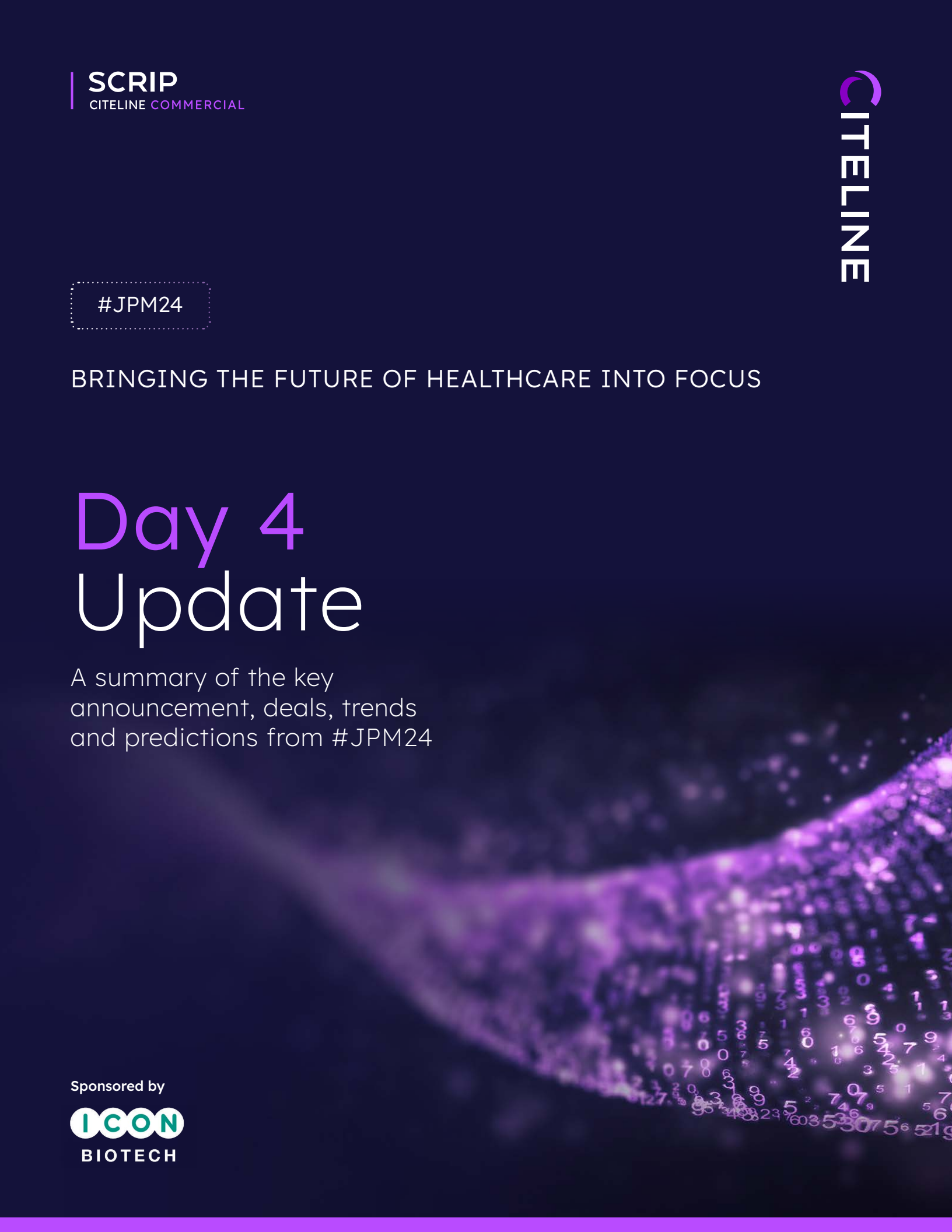

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J.P. Morgan Day Four: Roche Sees Lots Of Partnering Interest, While Bluebird, Sage Offer Launch Updates

11 Jan 2024

Executive Summary

Daily notebook from the J.P. Morgan Healthcare Conference: Roche expects continued high interest in partnering; Vir explains cost-cutting gives it a stronger base; bluebird provides Lyfgenia performance to date; Sage details Zurzuvae launch trajectory; and Madrigal lays out next steps in NASH.

Roche Sees No Slowdown In Partnering Demand

Roche Pharmaceuticals CEO Teresa Graham told Scrip in an interview at the J.P. Morgan Healthcare Conference that she expects no change in demand from small to mid-sized biopharma companies seeking partners even as financial market conditions begin to improve.

“Last year, we saw fewer companies IPOing, we saw the amount of money being raised being lower, but you did see sort of an increase in partnering,” Graham said. “Even when you control for the big M&As that happened, you did see more companies looking to seek partnerships. Valuations for good companies are starting to creep back up, but I still am sensing, at least at this J.P. Morgan, that there's still a lot of interest in finding partnerships.”

In particular, for indications in which companies need to conduct large clinical trials to show a significant effect on a disease, small to mid-sized are looking for help from a big pharma like Roche Holding AG to make sure they conduct the right trial in the right way.

“And then, if you want to commercialize your drug outside the US, most small to mid-size companies have absolutely no ability to do that,” Graham said.

Roche operates in 100 countries and almost every therapeutic area, with manufacturing, regulatory, pricing and reimbursement, and commercialization expertise across most markets and indications. “Bringing that combination to the table when we seek partners, it's beneficial for us and it's super beneficial for that partner who may have a really great molecule but maybe not all of the resources that it needs to bring it to patients,” Graham said.

Even with the ability for companies to launch initial public offerings and raise other new funding in 2024 potentially improving, she said those fundamental elements will remain a challenge for smaller companies developing drugs for large indications across multiple global markets. (Also see "Finance Watch: J.P. Morgan Conference To Offer Glimpse Of 2024 Funding Forecast" - Scrip, 5 Jan, 2024.)

“You have to be pretty well capitalized in some of these areas to be able to bring something through on your own,” Graham said. “And the IPO market is just not seeming like it's going to get you there, so you're either going to get acquired or you're going to partner and it just a little bit depends on what the desires of that particular entity are."

Vir Reassures Cost Cutting Is From A Position Of Strength

Vir Biotechnology, Inc. CEO Marianne De Backer used J.P. Morgan as a platform to reassure investors that the company is on solid financial and scientific footing, despite a cost-cutting program announced in December and the failure of its flu candidate in Phase II last year.

“We are actually a company that is in really good shape,” De Backe said in an interview. “The reason why we announced the reorganization in December was not because we were a company in trouble, like you’ll see so many others, but because we thought that our geographic footprint didn’t make sense.”

Indeed, Vir ended the third quarter with $1.7bn in cash and equivalents, a wealthy cash balance for a biotech.

"We're a small company with 600 people, and we had four sites, three sites in the US, and we just wanted to streamline that and make us a little more fit for purpose." The company announced a plan to cut 75 positions, or 12% of its workforce and close two R&D sites as part of a cost reduction program aimed at saving $40m per year. (Also see "Vir Biotechnology Decreases The Surplus Population" - Scrip, 14 Dec, 2023.)

The company is expecting important data readouts this year for its hepatitis B and D programs, including Phase II data from its Phase II SOLSTICE trial evaluating VIR-3434 and VIR-2218 as monotherapy and in combination for the treatment of hepatitis D in the first quarter of 2024, and in hepatitis B by the end of the year.

De Backer plans to use Vir's cash stockpile to fund the development of those current programs and Vir's earlier pipeline, but also expand through dealmaking.

"We also have the opportunity to look at some external innovation," she said, noting the company is interested in assets that are in early clinical development or close to entering the clinic.

Bluebird Sees Fast Treatment Center Response To Lyfgenia Approval

Bluebird bio already had a significant head start on the commercialization of its sickle cell disease gene therapy Lyfgenia (lovotibeglogene autotemcel) when it was approved on 8 December, with 40 qualified treatment centers (QTCs) established to deliver the company's previously approved Zynteglo (betibeglogene autotemcel) for transfusion-dependent beta-thalassemia and 27 of those sites ready to administer Lyfgenia. (Also see "Vertex/CRISPR Nab First-Ever Gene Editing FDA Nod, Overshadow Bluebird's Same-Day Win" - Scrip, 9 Dec, 2023.)

Just one month later, the company announced at the J.P. Morgan Healthcare Conference that there are now 48 QTCs ready to administer Zynteglo, 35 of which are able to handle sickle cell patients accessing Lyfgenia. Bluebird expects 85-105 patients to begin the treatment process with one of its gene therapies in 2024, including Zynteglo, Lyfgenia and Skysona (elivaldogene autotemcel) for children with cerebral adrenoleukodystrophy (CALD). The company said 26 patients started treatment with one of its products in 2023, including 20 for Zynteglo and six for Skysona.

Tom Klima, bluebird's chief commercial and operating officer, told Scrip in an interview during J.P. Morgan that the FDA's approval of Lyfgenia about two weeks ahead of its expected 20 December action date was a motivating factor for more QTCs to come online and to ready their sites for treating patients with Lyfgenia.

"I think when some of the centers saw the early approval, that really motivated them to move faster," Klima said. "I also think that there was a lot of concern, at least by investors, around the boxed warning for Lyfgenia. And their concern was that maybe some physicians wouldn't want to use something with a boxed warning, which is kind of strange to say because most everything a transplanter uses has a boxed warning. But the fact that eight or nine more centers came on after we got the label, I think just speaks right to that fact."

He said the fast pace of new QTCs readying for Lyfgenia administration “also speaks to the unmet need. The patients who have sickle cell disease are either in these QTCs or looking for gene therapy.” Klima said there is “a huge misperception out there that patients who have sickle cell disease won't come forward and want to be treated with gene therapy,” but noted that “I don't think a qualified treatment center would take the time or the effort to come on board unless they had patients to treat."

Bluebird expects even more QTCs to come online as the year progresses. The firm also reported some progress on the reimbursement side, with a second outcomes-based agreement in place that expands coverage to 200 million covered lives.

Sage Optimistic About Zurzuvae Based On Early Days Of Launch

Sage Therapeutics, Inc. is prepared to turn its newly approved Biogen, Inc.-partnered Zurzuvae (zuranolone) into a blockbuster product based on its postpartum depression (PPD) indication alone and is optimistic about the drug’s commercial prospects based on the early days of its launch, CEO Barry Greene told Scrip in an interview during J.P. Morgan.

The US Food and Drug Administration approved Zurzuvae for PPD in August but issued a complete response letter (CRL) for major depressive disorder (MDD), saying that another pivotal trial was needed to clear the positive allosteric modulator of the GABA-A receptor for MDD, a much larger indication. The drug required Drug Enforcement Administration scheduling, so it did not launch for PPD until mid-December.

"I believe that Zurzuvae is the key to unlock the blockbuster potential in PPD and help many, many women suffering from PPD,” Greene said.

He noted that one out eight women who deliver babies in the US suffers from PPD, or about half a million women annually. Sage is optimistic about Zurzuvae’s prospects to treat many of these mothers because of the response the drug has seen pre- and post-launch in terms of media coverage of Zurzuvae and PPD that have helped to destigmatize the disease, inclusion of Zurzuvae in treatment guidelines adopted by the American College of Obstetricians and Gynecologists (ACOG) and pre-Christmas prescribing activity for Zurzuvae.

“We made the drug available December 14 and so that gave us about 10 days where [doctor] offices were open and moms could access their health care provider,” Greene said. “In those 10 days, we saw a robust response from the medical community. We saw moms getting diagnosed and Zurzuvae being written – and not only by psychiatry, but Zurzuvae being written by OB/GYN and primary care – so, we're really enthusiastic in the early days of launch. Our goal going forward is to establish Zurzuvae as the frontline treatment of women suffering from PPD.”

Both Sage and Biogen have sales teams in the field talking to doctors about PPD and an omnichannel effort is under way to provide digital education about Zurzuvae. Payers also have been receptive to Zurzuvae, Green said, finding the product attractive because it treats a serious medical condition with a two-week regimen rather than a chronic medication. So far, the only utilization management tool that some payers are implementing is a requirement for a physician attestation that the patient has PPD and not some other form of depression, he added.

As for whether or not Sage and Biogen will pursue further development of Zurzuvae for MDD, running another Phase III clinical trial in the larger indication is not on the near-term horizon.

"We're solely focused with Zurzuvae on PPD," Greene said. "If there are any changes to MDD or we make decisions about MDD, we will update, but people should be focused on Zurzuvae for PPD only."

Madrigal Will Focus On NASH With Advanced Fibrosis, Regardless Of Label

Madrigal Pharmaceuticals, Inc. is preparing for its 14 March US Food and Drug Administration action date for resmetirom and gave a clear outline at J.P. Morgan of which patients it intends to focus on if, as expected, the THRβ agonist becomes the first approved drug therapy for non-alcoholic steatohepatitis (NASH). CEO Bill Sibold told a 10 January session that Madrigal will market resmetirom as a product for NASH patients with advanced fibrosis even if it gets a broader indication including earlier-stage patients. (Also see "Madrigal Shows Confidence As It Awaits FDA's March Decision On Resmetirom" - Scrip, 13 Nov, 2023.)

The company estimates a diagnosed US NASH population of 1.5 million at launch, and expects slightly more than one-third – 525,000 – will have advanced fibrosis, meaning a fibrosis score of F2 or F3. A patient with liver cirrhosis is considered F4, while F0 and F1 scores mean no or early-stage fibrosis. Of that 525,000, Madrigal intends to focus on an estimated 315,000 patients who are under the care of targeted specialists focusing on hepatology or gastroenterology, Sibold said.

"From our perspective, since we aren't going to be calling on primary care physicians, etc., if the patient isn't being treated by a specialist who understands the disease and the product, then they won't be getting the drug because that's who will be prescribing," the exec said. Madrigal will not look to treat earlier-stage NASH patients, he clarified, and is currently recruiting a 700-patient, 36-month Phase III outcomes study that might provide data to support adding cirrhotic patients to the product label.

Sibold said it is still too early to get specific about pricing but he talked about the product as a “specialty launch that will be specialty pricing.” The exec also pointed to the Institute for Clinical and Economic Review’s analysis last February that determined that resmetirom would meet cost-effectiveness thresholds if priced between $39,600 and $50,100 per year. (Also see "ICER: Madrigal’s Resmetirom Looks More Cost-Effective In NASH Than Intercept’s OCA" - Scrip, 16 Feb, 2023.)

“We see ourselves as becoming the first foundational therapy for NASH with significant fibrosis,” Sibold added, explaining that he thinks resmetirom might be the backbone of combination therapy as more NASH drugs with different mechanisms of action gradually enter the market.

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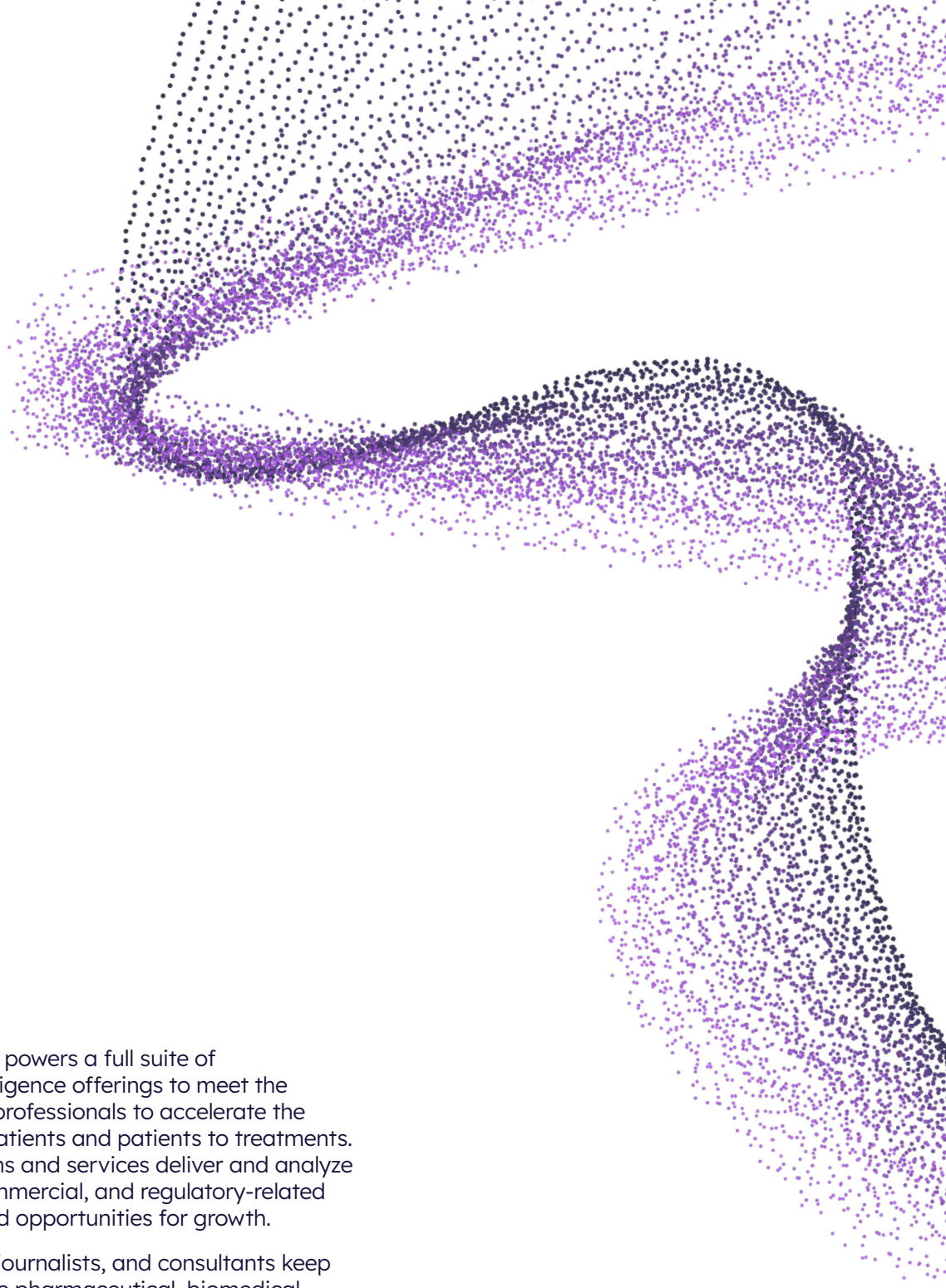

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