Exa-cel training simulations align teams for launch

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The final phase of mock operations at Vertex Pharmaceuticals headquarters in Boston, MA.

As the launch of our gene-edited therapy for the treatment of sickle cell disease and beta thalassemia, exa-cel, approaches later this year, our teams managing the commercial launch readiness process are hard at work. Global teams aimed to be launch-ready by August 1, meaning every step of the end-to-end patient experience for exa-cel had to be ironed out by then. In preparation, the teams needed to perform “mock operations,” a series of simulation exercises to practice procedures and test the software systems required for the exa-cel treatment journey once the product is approved by a regulatory authority.

> September is Sickle Cell Awareness Month! Download the Zoom background attached to this page and stay tuned for more content all month long. Also, as we go ***#fullstHEMEahead*** to prepare for not one, not two, but potentially three regulatory approvals later this year, we've created the **Exa-cel Launch Readiness Hub** **Vnet site** to act as an exa-cel launch readiness hub. Click **here** for the latest exa-cel launch updates, patient stories and to learn more about each **step along the journey**.

Previous activities included organizational meetings and a **tabletop exercise** that @Mark Bradley, Director of Global Commercial Strategy, likened to script reading. Over three days, cross-functional teams from the Boston and Paddington offices talked through the commercial process for exa-cel step-by-step and anticipated any potential issues that could arise.

Upon approval, the exa-cel end-to-end commercial process will entail many steps. A patient's blood stem cells will be collected and sent to a manufacturing facility where **CRISPR/Cas9** gene editing is used to edit the hematopoietic stem cells to **produce fetal hemoglobin**. The edited cells are shipped back to the authorized treatment center and patients will be **prepped for transplantation**. Patients will then be infused with their edited stem cells and receive regular post-treatment checkups.

The initial mock operations meetings clearly defined the scope and process of the exa-cel launch, but now it was time to put these plans to the test. The exercise represented the final phase of mock operations — a live rehearsal that occurred in two parts. The first activation covered every step from order initiation to infusion **for six simulated patients**. The teams needed to practice the standard procedures, but also exceptions like rescheduling a patient appointment or hiccups in the shipping process.

Last month, global teams completed the second half of mock operations for the impending exa-cel launch. Functions from Commercial Manufacturing and Supply Chain, Quality Assurance, DTE, Finance US Market Access, Commerical Strategy, International Commerical, and US Heme Business Unit all came together for the global exercise, said @Gabby Manoff, Global Commercial Strategy Senior Manager. Mark and Gabby spearheaded the planning for mock operations across multiple functions and global sites.

This exercise focused on pressure testing the new software systems Vertex data technology engineers designed to accurately track the extensive exa-cel patient journey in a user-friendly

manner. "The objectives were to first and foremost confirm that the commercial process works. Then, we needed to confirm that our systems were alive and working the way we expected them to, and that our teams were trained and able to handle unexpected scenarios," said Mark.

Software is needed to record every step in the planned commercial process, from manufacturing and ordering to data analysis and regulatory affairs. Several DTE systems in the IMPACT (Integrated Management of Patient Access to Cell Therapies) program support the exa-cel journey, including the Vertex Connects Order Management Portal (the "Vertex Connects Portal") and Oracle. The Vertex Connects Portal is an online system that will manage the exa-cel ordering process such as scheduling drop-off and pick-up times, track chain of custody and printing supply labels. The enterprise resource planning suite, Oracle, will track the manufacturing and processing of exa-cel before it goes to authorized treatment centers.

Building the Vertex Connects Portal for exa-cel took two years, and this was the first opportunity that commercial teams had to test it in real-time, added Mark. "The slogan was 'our first patient isn't our first time doing this.'"

Participants drew on their respective expertise and some from prior launch experiences to guide the planning and execution. "We were all eager to get into it, test the process, and learn," added Gabby.

"It's interesting because this is not just a new launch for Vertex but also relatively new in the industry. There are only so many cell and gene therapies, and we will hopefully be the first CRISPR therapy," said Gabby. "It's not like this is a tried-and-true tactic that everyone does across every company — it's a new thing that we ran with."

Over three days, teams practiced the procedures and handoffs required in the exa-cel launch. Challenges arose almost immediately, said Mark, and they had to adjust the schedule to ensure all participants were competent and comfortable with the standard procedure before moving into more complex scenarios.

"For me, it brought home how important it is to do the real thing," said @Stephanie Hecht, Marketing Associate Director, International Heme Business Unit Management, who represented the international teams. "It's the first time that it all came together, and commercial leads really appreciated and understood the complexity of the treatment process."

Despite these challenges, Mark and Gabby agree the team came together to troubleshoot calmly and efficiently. “I feel like we acted as one Vertex team,” said Gabby. She assessed the success of the mock operations exercise through surveys taken before and after. Participants were asked about their comfort levels with all aspects of the exa-cel launch, from handoffs to system management.

The results exceeded the team’s expectations, showing double-digit increases in participant comfort levels across all categories after completing the exercise. Teams left the mock operations feeling significantly more confident with steps required in the exa-cel patient journey. “I think everyone realized that we do know what we're doing. We are launch-ready."

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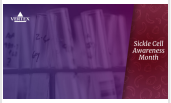

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