José F. Rodríguez-Orengo – CEO, MBQ Pharma

Puerto Rico has the talent, infrastructure, and expertise to deliver world-class research

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Could the future of metastatic cancer treatment lie in a groundbreaking compound developed in Puerto Rico? Dr. José F. Rodriguez-Orengo, CEO of MBQ Pharma, leads the development of MBQ-167, a first-in-class therapy designed to disrupt cancer progression. As the company navigates challenges in funding, it is also paving the way for Puerto Rico to emerge as a global biotech hub.

How has your role evolved since transitioning from CEO of FDI Clinical Research to leading MBQ Pharma, and how do you balance the demands of biotech, CRO, and academic work?

Balancing my responsibilities requires surrounding myself with exceptional individuals—people who are not only highly skilled but often more capable than I am in their respective fields. My focus is to guide their efforts, ensuring the team operates cohesively and that all aspects of our work remain aligned with our goals. At MBQ Pharma, we've embarked on a transformative journey to position Puerto Rico as a hub for research and innovation, moving beyond its traditional focus on manufacturing. In 2018, my university colleagues identified MBQ-167, a compound with the potential to redefine cancer treatment. We began, through MBQ Pharma, by licensing the patent from the University of Puerto Rico, where it was developed, and securing initial funding from close supporters who believed in our vision.

While our aspiration was to carry out the research locally, limitations in infrastructure required us to collaborate internationally, involving partners in the United States, Canada, and China to conduct

preclinical studies. Under the leadership of our former CEO, Dr. Federico Goodsaid, we compiled a comprehensive data package that led to FDA approval in 2022 to advance to a Phase 1 clinical trial. This milestone is historic, marking the first time a compound originating from research at the University of Puerto Rico has reached clinical testing.

Our immediate focus is on breast cancer, one of the leading causes of cancer-related deaths globally, including in Puerto Rico and the United States. Although prevention and early diagnosis are critical, the prevalence of breast cancer is expected to rise by 2-3% annually through 2030, particularly among younger women. These cases tend to be more aggressive and metastatic, demanding rapid and effective interventions. MBQ-167 represents not only a critical step forward in addressing these challenges, but also a broader breakthrough for Puerto Rico's research ecosystem. By demonstrating the feasibility of developing innovative therapies locally, we hope to inspire and pave the way for other researchers to follow, creating a foundation for future advancements that will benefit patients worldwide.

What is the scientific basis of MBQ-167, and why does the simultaneous inhibition of Rac and Cdc42 represent a breakthrough in cancer treatment?

MBQ-167 is rooted in over 25 years of research into the mechanisms driving cancer progression, specifically focusing on critical signaling pathways. Traditional approaches in oncology have often targeted the RAS pathway at the cell membrane or cyclins within the nucleus—two key points in the cancer signaling process. However, these strategies have faced challenges, such as resistance arising from RAS mutations, which allow cancer cells to evade treatment, proliferate more aggressively, and lead to faster recurrence.

MBQ-167 takes a novel approach by intervening at the midpoint of this pathway, targeting Rac and Cdc42, two Rho GTPases that play a pivotal role in cancer cell migration, invasion, and metastasis. By inhibiting these molecules simultaneously, the compound disrupts the signaling network that drives metastasis and tumor growth. Unlike therapies that block signaling at the start or end of the pathway, MBQ-167 acts as a critical barrier in the middle, effectively halting the progression of cancer.

Preclinical studies have demonstrated the compound's remarkable efficacy. In animal models, MBQ-167 not only prevents early-stage metastasis but also stops the spread of established metastatic cancer when combined with other therapies. This dual action significantly reduces tumor growth and halts metastasis, even in advanced stages. As a first-in-class compound, MBQ-167 represents an unprecedented advance in oncology. No other pharmaceutical company or research team is currently pursuing this specific approach, underscoring its innovative nature. While the initial focus is on breast cancer, the potential applications extend to other solid tumors, such as ovarian cancer, where Rac and Cdc42 are also expressed. By addressing these fundamental drivers of cancer progression, MBQ-167 offers a transformative strategy that could reshape the landscape of cancer treatment.

What are the next milestones for MBQ-167 in breast cancer trials, and could its development extend to other therapeutic areas?

MBQ-167 is making significant progress in its Phase 1 clinical trial, with the second of eight planned cohorts completed in January. The safety committee meeting is scheduled for early February, and we anticipate starting the third cohort immediately. Each cohort involves a gradual increase in

dosage, with the first beginning at 20 mg twice daily, the second doubling to 40 mg, and the third set to escalate to 80 mg. This stepwise approach, required for a first-in-class compound, is designed to evaluate the safety as dosages rise.

Initially, our goal was to conduct the trial entirely in Puerto Rico, where the prevalence of breast cancer offers a valuable patient base. However, recruitment proved challenging. To address this, we expanded the trial to include sites in California, Tennessee, and Florida. This move greatly accelerated patient enrollment; once regulatory and Institutional Review Board (IRB) approvals were secured, recruitment at these four locations was completed within a week. A key finding from the first cohort was that plasma levels of MBQ-167 were higher than predicted by preclinical models, suggesting robust systemic absorption. While no toxicity or adverse effects were observed, the low initial dose did not produce measurable therapeutic effects, likely due to the advanced disease stages of the participants. As the dose increases in subsequent cohorts, we are optimistic about detecting signs of efficacy.

One of the most notable advantages of MBQ-167 is its excellent safety profile. Unlike traditional chemotherapies, which are often associated with severe side effects such as hair loss and fatigue, MBQ-167 has demonstrated no toxicity in preclinical studies. Its formulation as an oral capsule further enhances its appeal, allowing patients to take the medication at home, significantly improving convenience and quality of life. Participants are monitored over an initial 21-day period, with the option to continue treatment for up to 56 days if no adverse effects are observed. Comprehensive assessments of cancer progression and metastasis are conducted at the start and at the 56-day mark using CT imaging techniques. Our first patient in the second cohort is approaching this milestone, and preliminary indications suggest the drug is well-tolerated.

While our primary focus is on breast cancer, the mechanism of action of MBQ-167—targeting Rac and Cdc42—has the potential to address other solid tumors as well. This groundbreaking approach positions MBQ-167 as a promising candidate for broader applications in oncology, paving the way for significant advancements in cancer treatment.

How has funding supported MBQ Pharma’s growth, and what strategies are you employing to advance clinical trials and explore additional cancer research?

Funding has been instrumental in MBQ Pharma’s evolution, underpinning our transition from foundational research to clinical trials. The Puerto Rico Science, Technology, and Research Trust (PRSTRT) played a critical role early on, providing administrative support for patent licensing and financing for pre-clinical experiments for MBQ-167. This early backing not only established our credibility but also positioned us as a leading example of the PRSTRT’s Advanced Research Grants Program, a distinction highlighted by its CEO, Lucy Crespo.

We are now focused on leveraging additional funding opportunities, including PRSTRT’s matching program, which has the potential to double contributions from private investors, such as the $250,000 we received from the Popular Impact Fund. While much of our initial investment efforts have been centered in Puerto Rico, we are expanding outreach to the East and West Coasts of the United States to secure the larger-scale funding necessary for long-term development. In 2023, we secured a $4.4 million grant from the Department of Defense to support clinical trials, a milestone that ensures the completion of our Phase 1 study. However, we are actively preparing for a pre-Series A funding round to raise an additional $5 million, which will enable us to advance beyond the current phase and explore further applications of MBQ-167.

One of our most promising avenues of research is pancreatic cancer, a disease with limited

treatment options and a high mortality rate. Preliminary studies suggest that MBQ-167 may effectively inhibit metastatic pancreatic cancer, similar to its demonstrated efficacy in breast cancer. This potential breakthrough has driven us to pursue both local and federal grants alongside private investment to expand our research into this critical area. Our extensive experience in academic grant writing has proven invaluable, allowing us to secure non-dilutive funding while building investor relationships to support our broader vision. By combining a strategic approach to financing with innovative science, we are confident in our ability to push boundaries and make meaningful advancements in oncology research.

What challenges and opportunities shape the development of a biotech company in Puerto Rico, and how does the local investment landscape influence progress?

Establishing a biotech company in Puerto Rico presents both promising opportunities and notable challenges, particularly in terms of investment dynamics. One of the primary hurdles is the limited awareness of the biotech sector among local investors, who are predominantly focused on technology ventures. Biotech requires a deeper level of expertise and understanding, and the absence of this knowledge often leads to hesitancy. Investors tend to favor familiar areas, leaving innovative fields like biotechnology underfunded and underexplored.

At MBQ Pharma, while we are fully focused on advancing our Phase 1 clinical trial, our broader vision includes conducting additional preclinical studies and refining protocols for regulatory submission to the U.S. Food and Drug Administration (FDA). However, these efforts remain constrained by funding limitations. I am confident that as we generate compelling clinical data that validates our hypotheses, we will attract more interest and shift perceptions about the viability of biotech investments in Puerto Rico. The cautious approach of local investors is understandable given their success in the technology sector and the lack of experienced advisors to guide them into the complexities of biotechnology. Addressing this requires not only showcasing robust scientific results, but also cultivating partnerships with experts who can provide the necessary counsel to bridge the knowledge gap.

Despite these challenges, the opportunities in Puerto Rico’s biotech sector are substantial. The island is steadily gaining recognition as a burgeoning hub for research and development, offering a platform for innovation and commercialization. At MBQ Pharma, we aim to position Puerto Rico as a competitive force in the global biotechnology industry, demonstrating the transformative potential of local innovation and encouraging investors to embrace the future of this dynamic field.

How has Puerto Rico’s clinical research landscape progressed, and what initiatives are improving accessibility for trial participants?

Puerto Rico’s clinical research landscape has seen notable advancements, with FDI Clinical Research currently conducting 53 active studies spanning all trial phases, from Phase 1 to Phase 4. While some sponsors initially prefer to keep studies within their primary clinical sites, many return to Puerto Rico after experiencing the exceptional quality and efficiency of research conducted here. To further enhance accessibility and participation, we recently opened a second clinic in Mayagüez, a region historically underserved in clinical research. This expansion addresses a significantchallenge for participants who previously had to travel long distances to San Juan, often dedicatingan entire day for weekly visits. By bringing clinical trials closer to participants, we not only alleviatelogistical burdens but also improve the overall trial experience, reinforcing Puerto Rico’s position asan emerging leader in the global clinical research field.

What is your vision for MBQ Pharma’s future, and what sustains your passion for this work?

My vision for MBQ Pharma is to evolve into a leading pharmaceutical company rooted in Puerto Rico, with a mission that extends beyond oncology to embrace a broader portfolio of innovative therapies. Leveraging our growing expertise, we aim to identify and license promising products developed by researchers both locally and in the United States. The knowledge we have accumulated thus far positions us to streamline processes and address barriers more effectively, enabling faster and more efficient development of therapies that can ultimately reach the market.

On a personal level, this work fulfills a dream I have nurtured since beginning my research career at the age of 18. My nearly three decades at the University of Puerto Rico provided the foundation for this journey, and I am deeply committed to giving back to the institution that shaped my path. By channeling revenue from successful products back into the university, we can contribute to its growth and sustainability, creating a lasting impact.

What is your final message for your global readers?

To a global audience, I want to emphasize that Puerto Rico has the talent, infrastructure, and expertise necessary to deliver high-quality research and innovation. In the months ahead, we aim to validate this on a broader stage. Breast cancer remains a critical challenge, claiming the lives of approximately 45,000 women annually in the United States alone—equivalent to 9 lives lost every hour. Our ultimate goal is to reduce this devastating toll, and we remain fully committed to achieving meaningful change through our work.

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