September 4, 2025

Update on SL1009 (Sodium Dichloroacetate Oral Solution, DCA) for the Treatment of Pyruvate Dehydrogenase Complex Deficiency (PDCD)

Dear Mitochondrial Disease Community,

We are writing to share an important update regarding SL1009 (DCA), our investigational therapy for the treatment of PDCD. Recently, Saol received a Complete Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) regarding our application for approval of SL1009 (DCA). The FDA informed us that our New Drug Application cannot be approved in its current form and outlined specific observations that we must address before potential approval can be reconsidered. To address these observations as the FDA requested it would take several years and millions of dollars.

While this decision was not what we had hoped for, our near-term objective is to find a faster path to approval and maintain access to SL1009 through the ongoing open-label extension of our clinical trial and the Expanded Access Program. Our progress with the FDA will impact our ability to continue to provide SL1009.

We want to reassure you of two important things. First, we remain committed to advancing SL1009 toward potential approval. Second, our team will work closely with the Agency to understand their feedback and define the clearest and fastest path forward. We remain encouraged by the four years of clinical-data we have captured through the double-blind placebo-controlled portion of the trial and the open-label extension. We feel strongly that SL1009, with its established safety profile, could be an important treatment option for patients with PDCD.

As many of you know, developing and reviewing therapies for ultra-rare diseases presents unique challenges. We are encouraged by recent actions and commentary from the FDA that acknowledge the need for more flexible and faster pathways for review and approval of rare and ultra-rare diseases, like PDCD, where there is a life-threatening unmet need.

We are deeply grateful to the PDCD and broader mitochondrial disease communities. Patients, families, clinicians, and advocates have provided steadfast partnership and support. From clinical trial participation, advocacy, and education, your efforts have been critical in bringing this therapy forward. We plan to keep you informed as we work through this process.

Lastly, we assure you that we will work tirelessly and constructively with the FDA to work towards an approval as soon as possible. Your help would be greatly appreciated. We remain hopeful and committed to bringing forward the first approved therapy for PDCD and to ensuring that the voices of rare disease communities are heard in shaping a more equitable regulatory path.

With gratitude,

Dave Penake
Chief Executive Officer
Saol Therapeutics