Where does the cell and gene field need to go to thrive? Critical considerations as you move your programs forward.

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The gene and cell therapy world today is vastly different than the world we were living in a few short years ago. Of course, some fundamental aspects remain unchanged – paramount among them, there remains a massive potential to have a positive impact on patients, families, caregivers, and our overall health care system. However, some critical aspects of our industry have changed significantly. Just a few years back, suboptimal product quality standards were accepted in order to deliver transformative products to patients that desperately needed them. This risk/benefit profile was warranted, and these products have positively impacted and saved countless lives. Consequently, the field has also observed a number of patient safety issues that are directly related to suboptimal product quality in programs currently in the clinic moving towards commercialization. These programs will require solutions before they proceed any further and new programs need a better path from the start.

Recent advancements in technology allow us to make step changes in the quality of gene and cell therapy products while also delivering products under the timelines that patients are hoping for. These technological advancements have helped to shift the risk/benefit calculation for both regulators and patients. We no longer need to accept suboptimal product quality in order to deliver these gene and cell therapies to patients. As we look to provide solutions for more common diseases, the importance of product quality and safety will be even more critical.

As a collective industry, we have dramatically improved our approach to analytical testing, starting materials, nucleic acids, and cell lines, and developed elegant manufacturing processes that prioritize driving down product impurities and producing functional full capsids. Regulators are also willing to work with product developers to implement next generation processes for products at all stages of development. Because of these improvements, product developers should only be satisfied with a CMC approach that delivers what patients deserve and regulators expect.