



# ISCT TALKING WITH GIANTS

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Cell & Gene Therapy®

## 5 Questions with Robert Preti, PhD

From the very beginning, Robert “Bob” Preti had a penchant for inspiring others. He began in the classroom, teaching as an elementary and secondary teacher, then as a professor at New York City area colleges. Bob received a Bachelor of Science degree in biology from Fordham University, and a Master of Science degree and Doctorate, both in biology, from New York University. He held several roles with the New York Blood Center from 1990 to 1997, including tissue bank director, director of hematopoietic stem cell processing, scientific director and associate investigator. From 1996 to 1999, Bob was the director of hematopoietic stem cell processing and applied research at Hackensack University Medical Center in Hackensack, N.J. He also worked as a research scientist for Advanced Tissue Sciences, where his work led to the Dermagraft product currently marketed by Organogenesis. This interesting career path led Bob to co-founding a contract development and manufacturing organization, the highly innovative PCT Cell Therapy Services, in 1999, which he has led for its now twenty-year history. After PCT’s acquisition by Hitachi Chemical in 2017, Bob’s role expanded as he became responsible for development, management and oversight of the global business operations of Hitachi Chemical’s regenerative medicine business unit as its General Manager. In addition, he has served in a leadership capacity for many professional organizations, including treasurer and founding member of the International Society for Cellular Therapy (ISCT). He recently completed a five-year term as a director for AABB as well as a two-and-a-half-year term as Chairman for the Alliance for Regenerative Medicine, where he previously served as Vice Chairman and co-chaired the Standards and Technology Committee. He has published and presented extensively on a variety of topics related to cellular therapies.

### 1 How is the cell and gene therapy field different now compared to when you started?

When I started in the field in 1986, there really was no cell and gene therapy industry at all. There was no regulatory framework, no real investment backing, no enabling services, no reimbursement mechanism. Some people saw the potential of how cells can be leveraged for treatment. It seemed like a natural extension of graft manipulation and bone marrow processing.

Over the years a variety of efforts have really moved the industry forward, like partnerships with regulatory agencies. The FDA took a leading role in the early 1990s, when they noted that it was drug development work and not just bone marrow transplants anymore. Now, the regulatory framework is maturing and it guides the development of these products worldwide.

We were asking questions early on about the product itself and the mechanism of action, trying to better understand the cells so that we could build better products. While we’re still asking those questions, what has changed is we’re thinking of this as manufacturing with a focus on achieving commercial production.

### 2 Where do you see the cell and gene therapy field in 5 years?

I can speak to it from a manufacturing standpoint. We have always viewed the development and commercial distribution of these products as relying on four key factors: quality (robust production of products), cost of goods, scalability and sustainability. We’ve made strides in all of those areas but we’re still, by and large, operating on manual systems that are very expensive and have quality issues. Over the next five years, I see many of those challenges being knocked down so that that prospects of commercial viability can be sharpened.

Payment models are getting more sophisticated. Right now, as each company gets close to commercialization,

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they’re working out different, creative payment models with the payers. For now, we’re able to get these therapies out the door, but those models are going to have to be much more sophisticated for larger scale distribution. This must be more broadly discussed as an industry.

This is truly a global industry now. Completing the globalization and convergence of international regulatory systems is something that will happen over the next five years. We’re going to have to find a common, harmonized regulatory pathway around the world so that companies developing in one part of the world can consider their products being developed in other parts of the world, knowing that what defines quality from a regulatory standpoint should define quality in any other region.

### 3 Why are you so passionate about working in the cell and gene therapy field?

We could change the way medicine is delivered and actually start to approach cures by going after the very root of the problem as opposed to treating symptoms. From the very beginning, I found that concept incredibly exciting. I’ve always lived my life by the phrase that’s credited to William James which is, “The great use of life is to spend it for something that outlasts it.” I couldn’t imagine better use than by being a part of creating the fourth pillar of medicine. Clearly, the effects of this work will outlast my life.

### 4 What are the biggest challenges facing the development of new cell and gene therapies?

The central challenge we face is the difficulty in completely defining mechanism of action, product identity and potency. Any changes we make in the collection, manufacturing, storage or transportation of the cells all have the potential to change the potency and safety profile of the product. This puts a lot of pressure on the development stage. We focus on more discreet

measurements we can make during manufacturing, so that by extension the final product is comparable.

Another is achieving deliverability, which we define by that profile I mentioned – cost, quality, scalability and sustainability. We have to look at how the challenges have been solved in other industries like biologics. At one time, regulatory, scientific, and development frameworks didn’t seem to apply to biologics. But a convergence of all of the different enabling skills came together in that industry to solve these challenges. How do we leverage that in the regenerative medicine space and come up with our own creative solutions?

A focus of ours right now is the human resource challenge. Now there are not enough trained people to handle the highly manual nature of the industry’s manufacturing. This is compounded by the movement of labour throughout the industry. That will be partly solved as we begin to improve the manufacturing platforms to become less reliant on labour.

Another work stream, of course, is that we’ve got to figure out how to pay for the products. What’s the right way to reimburse for the tremendous amount of innovation that’s going on and the life-changing aspects of these medicines? We’ve got to establish a value-based reimbursement mechanism.

### 5 For people just starting out in this field now, what would be the one piece of advice you would give them based on your experience?

Keep the patient front and center and the mission stays clear. Understand that the industry is still in its relative infancy. There are many opportunities to make a difference if you really commit to solving those big challenges I mentioned. The opportunities to make a good living will be there – so be patient, as this industry is not going away. If you do that, you are sure to follow Williams James’ advice and spend your life on something that outlasts it.