

## Can lab grown tissues help test drugs for rare diseases?

**Marshall L. Summar, MD:** I am Marshall Summar, Chief of Genetics and Metabolism at Children's National Medical Center in Washington, DC, for Medscape Rare Diseases. I am at the National Organization for Rare Diseases (NORD) Orphan Products Breakthrough Summit with Jim Powers, the CEO of HemoShear (Charlottesville, Virginia). Jim's company is leading some of the technological revolutions in rare diseases, and we are going to talk about what is going on.

**James C. Powers:** It will probably help for me to describe what our basic technology is, and then talk about the rare diseases applications. Our company creates human tissue systems that accurately replicate human diseases in the laboratory. We still lack systems in the laboratory that accurately replicate human disease and that can predict whether a new drug candidate will work in a human. That is the problem that we are trying to address.

**Dr Summar:** You bring up an interesting point. We have been doing mouse studies for years, and we have found lots of ways to make mice better and fix mice. But what we have found is that biochemically and genetically, mice and humans aren't quite the same and don't behave the same. Does this provide a bridge that is a little closer to the human model? Is that what you are getting at?

**Mr Powers:** It absolutely does. You could ask, why study the disease in an animal at all if the animal doesn't develop the disease? That is certainly the case in rare diseases. It is hard to put a human rare disease in an animal. We believe that, to some extent, we can leapfrog animal studies and go straight to assessing biology in a human-relevant context using human tissue.

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