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### Translational Research for Autism Spectrum Disorder

Paul Rokuskie:

Autism Spectrum Disorder is one of the greatest medical and social challenges facing not only the United States but the entire world. The medical community is beginning to focus their resources on not only finding the cause but hopefully some viable treatment options for those diagnosed with this neurological disorder.

You're listening to ReachMD. I'm Paul Rokuskie, your host, and with me today is Dr. Robert Ring, Vice President and Head of Translational Research for Autism Speaks, the world's leading autism science and advocacy organization and the president of Delivering Scientific Innovation for Autism, better known by the acronym DELSIA.

Dr. Ring earned his BA double major in both fine art and biology from Westmont College in Santa Barbara, California and a Ph.D. in molecular neurobiology from City of Hope in southern California. He holds adjunct faculty appointments in the departments of psychiatry at Mt. Sinai School of Medicine New York and pharmacology and physiology at Drexel University College of Medicine Philadelphia.

Today we will be discussing translational research. Welcome Dr. Ring.

Dr. Robert Ring:

Hello Paul.

Paul Rokuskie:

Hello. So I gave a little bit of your academic background but if you could give us a little bit of your professional background as well.

Dr. Robert Ring:

Sure. Prior to joining Autism Speaks I was senior director and head of the autism research unit at Pfizer Worldwide Research and Development in Connecticut. This was the first dedicated research unit focused on developing medicines for neurodevelopmental disorders with a primary focus on autism, so a very unique opportunity for me. I had spent the majority of my career, over 10 years before that working at Wyatt, primarily in the areas of psychiatric and neurological medicines development.

Paul Rokuskie:

As head of translational research for Autism Speaks, a lot of people may not understand or know what translational research is. Could you fill our listeners in on that?

Dr. Robert Ring:

Sure. Translational research is a process that really is focused on converting breakthroughs in science and innovations in technology into applications or products that have real life benefit for families.

Paul Rokuskie:

So who are some of those partners?

Dr. Robert Ring:

Well, the translational research continuum, if you will, that extends from the proverbial bench to the bedside involves in the very early parts of the continuum, researchers in Academia at research institutions around the world, but it also involves the required partnership with the for-profit sector, with Biotech, pharmaceutical industry, entrepreneurs and small companies all working to really deliver those innovations into products that can be brought to market and help a variety of different unmet needs in the autism community.

Paul Rokuskie:

So can you provide any examples of research currently ongoing in translational research?

Dr. Robert Ring:

Well I think one area of translational research that many folks out there are quite familiar with and it represents an area of significant unmet need in autism is the area of medicines development. One of the real exciting things about autism research today is the current state of the science is very mature. We've learned an enormous amount about the underlying genetic risk architecture for autism. This has taught us a lot about the underlying biological mechanisms that are in play in the development of autism, which provide medicines developers with clues on how to develop therapeutic technologies to address the wide range of core and associated symptoms that define autism for individuals living with the disorder. And today we are seeing an increasing number of companies that have traditionally worked in the area of psychiatry and neurology who are actively exploring ways to leverage their investments in these areas to bring forth new medications to treat a variety of unmet needs in autism.

Paul Rokuskie:

So with your role at Autism Speaks, where do you see that organization's work with the industry?

Dr. Robert Ring:

Well Autism Speaks is in a very unique position to work across the entire partnered landscape involved in translation. We have traditionally been funding research in the earliest parts of the translational continuum through grants to academic researchers, but more recently, the foundation has been playing an active role in helping to facilitate the entry of companies into the translational process. This is a new area for the industry, the pharmaceutical industry, and we have been able to both help the industry understand the complexities of development but also make strategic investments that help to de-risk the landscape of investment for these companies. We really are playing a role of facilitator across this entire continuum.

Paul Rokuskie:

If you're just joining us, you're listening to ReachMD. I'm Paul Rokuskie, and I'm speaking with Dr. Robert Ring, Vice President and Head of Translational Research for Autism Speaks and the president of Delivering Scientific Innovation for Autism, better known as DELSIA. We're talking about translational research for autism spectrum disorder.

So we were just talking about where this translational research began, where it is today. Can you talk a little bit about where you see it in the next few years since this is a fairly new concept in the medical community?

Dr. Robert Ring:

Well, I see translation moving in a variety of directions in terms of product development to address unmet needs. And really what I mean by that is the state of the science and the direction of scientific research is really opening up a whole range of possibilities for new product development, not just medicines and therapeutic technologies, but much of the know how that's being generated today offers us the possibility of generating assistive technologies which can improve a variety of different daily living issues for individuals with autism. There's also real opportunity to leverage the science to develop diagnostics and other risk assessment tools which will enable earlier detection of autism. We know that early detection of autism helps to guide clinical care for these individuals and improve outcomes over time. So there's a variety of different product concepts that are out there and in play for the field of translation in autism.

Paul Rokuskie:

So not only in addition to being the Head of Translational Research for Autism Speaks, but you're also the president of Delivering Scientific Innovation for Autism, better known as DELSIA. Can you explain what your role in that capacity is?

Dr. Robert Ring:

DELSIA is an exciting new funding vehicle for us at the foundation. DELSIA is a venture philanthropy arm of the foundation. DELSIA compliments the very traditional investigator initiated grant mechanisms that we have been funding research for many years through, and it is a vehicle that allows us to work more explicitly with the for-profit sector. DELSIA funds companies, invests in companies at all stages of development and really helps to ensure that breakthrough science and innovations in technology that may be without capital are receiving the resources necessary to get them moving forward. It's just a sharper tool for us to influence the process of translation and allows us to impact other stages of the value chain.

Paul Rokuskie:

So have you been seeing a larger interest from the private sector because of the incidence rate that we're seeing dramatically changing within the landscape of the United States and around the world?

Dr. Robert Ring:

Absolutely. I think the epidemiology data that's coming out, the reported prevalence data is certainly opening the eyes of developers around the world. This is creating a massive landscape of unmet need, and with those numbers the market size for this need is

staggering. I think most companies that we meet with are quite surprised to see how large this market is and from a foundation point of view, we are happy if companies are able to make a financial return on their investment if at the same time, they're developing products that help create value for our community. So it's an exciting time for the evolution of translation and many of the products that are currently under development are going to define how care and daily living are managed in the future to come.

Paul Rokuskie:

So since DELSIA is just in its infancy, where do you see the future with that organization's role with working with industry?

Dr. Robert Ring:

I think DELSIA will develop a portfolio of investments in entrepreneurs and in small companies just getting going over the coming years that will really deliver a return on investment for the donors who provide us the resources at Autism Speaks, but a return on investment that's measured in progress, not necessarily in a financial return on investment. What we're trying to do through DELSIA is build a portfolio of companies that are working on a wide range of product concepts that are defining how autism will be managed and how daily living will be managed for individuals living with autism into the future.

Paul Rokuskie:

So I know recently Autism Speaks had their first annual investment conference that you participated in. So could you give us a little bit of background of who participated and what outcomes were after that conference was over?

Dr. Robert Ring:

Yes. The Autism Investment Conference was an inaugural event that was designed really to help bring together developers of new products, whether or not these are entrepreneurs or small companies, with the professional investment community. The business of translation is as important as the science of translation and without capital moving into the activity and supporting the activity, the ability to really move science forward to the marketplace will be lost. Recognizing this need we really saw the opportunity to play a role in helping bring private investors, professional investors from the venture capital world, private equity world, into a venue where we could showcase companies working on a wide range of product concepts that will ultimately define the marketplace in the future. So in that audience rather than a scientific conference, we had an audience filled with professional investors. We invited the insurance industry to come.

This is a product landscape that's going to define the future and it was a very successful meeting in helping create the network of interactions required to move things forward. There were a number of panels focused on different product areas. We've already talked about therapeutic technologies, but we also had discussion focused around housing and supports. There are a variety of different business models that are going to dominate the autism space, if you will, in the future and we look forward to having this as an annual event.

Paul Rokuskie:

So not only looking at the medicine portion and the medical side of the equation but also looking holistically at the entire landscape of how autism spectrum disorder affects individuals and families?

Dr. Robert Ring:

Oh, absolutely. As I said, there is a diverse opportunity landscape for business development and investment in the autism space, and it extends well beyond traditional areas of investment that is based on science like therapeutics and diagnostics. It also includes a rapidly emerging area of development of iPad applications and other support or assistive technologies, but also as individuals begin to age out of access to services and enter into adulthood, housing models and companies that are focused on putting adults with autism to work and placing them are all very exciting investment opportunities emerging out there.

Paul Rokuskie:

Is there anything else useful that you think our audience would need to know to understand translational research a little bit better?

Dr. Robert Ring:

I think the important take-home point is that translation and translational research is not a single discipline. It's a process that requires a wide range of expertise, a wide range of partners. Oftentimes with competing incentives and sources of funding, it's a process that requires a very strategic perspective in which to bring it forward and foundations like Autism Speaks I think are in a very unique position to help bring together the diverse set of partners required ultimately to move science from the laboratory setting to the marketplace where it's going to have an impact for families. You know, at the end of the day, there's no stack of scientific journal papers high enough to equal the impact that a new product on the market offers families, and as a foundation, just as any researcher out there, we're really looking at ways to help accelerate the process that moves that science to the marketplace. In order for us to be successful, we have to think beyond the science in order to deliver the science, and DELSIA represents one tool in our tool chest to help facilitate that.

Paul Rokuskie:

So where can our listeners go to learn more about what you've been talking about today?

Dr. Robert Ring:

Well I will always point your listeners to the Autism Speaks website [autismspeaks.org](https://autismspeaks.org). There's an enormous treasure trove, if you will, of resources available at our website. If you're a parent with a newly diagnosed child, we have our 100-day kit which helps parents navigate the complex array of resources and services needed at that point. We have a variety of other tool kits that help both parents, clinicians, teachers all along the way. It's an important website to visit.

Paul Rokuskie:

Well thank you very much to my guest, Dr. Robert Ring. Be sure to visit our website at [ReachMD.com](https://ReachMD.com) featuring podcasts of this and other series, and thank you for listening.