ANA 2018 Annual Meeting: Preview of a Landmark Year

Last year in San Diego, ANA Annual Meeting attendees were abuzz over the plenary on breakthrough therapies with antisense oligonucleotides. Seeing video of children with spinal muscular atrophy up and out of wheelchairs was nothing short of exhilarating. What will generate the most excitement this October in Atlanta? The latest advances in viral vectors for delivery of gene therapy? Advances in cell-based therapies?

M. Elizabeth Ross, MD, PhD, Chair of the ANA Scientific Program Advisory Committee (SPAC) and Director of the Center for Neurogenetics at Weill Cornell Medicine, offers a preview:

Q. Why is 2018 a landmark year in academic neurology?

During 2017-2018 we've had a number of major advances in therapeutics for neurological disorders. In our 2017 ANA meeting we presented breakthroughs in the use of antisense oligonucleotides for diseases like spinal muscular atrophy – a childhood form of ALS due to a single gene mutation. Conceived through fundamental understanding of how that gene works and the fact there is a cousin of the gene that can be co-opted into serving the lost function, this treatment is completely astonishing. Infants who would not have survived more than a few years and who would be in a wheelchair or respirator-dependent can now be independent and some of the earliest enrolled patients in the trial can walk. There are milder forms of the disease that affect adolescents and adults, and this treatment works for them as well. Other disorders that are being targeted using antisense oligos are in earlier stages of clinical trials but initial results are promising for CMT neuropathy, and even Huntington's disease.

In this year's annual meeting, we highlight cell-based therapies coming on board for neurological disorders.– We will be presenting results from early-stage clinical trials that use genetically altered stem cells from the patient, transplanting them back to the patient to reverse the deficit in a brain white matter disease of childhood, Adrenal Leukodystrophy (ALD). There are other cell-based strategies coming up for Parkinson's Disease, ALS and remarkable gene therapies that will be delivered with new types of viral vectors that more successfully reach the appropriate cells. There's a feeling of optimism in neurology as we anticipate having a range of new and highly effective tools for improving the lives of many individuals with neurological disorders.

Q Is there a theme to the meeting this year?

In each of our symposia, we focus not only on the latest advances in understanding what causes neurological disease but also on how that knowledge is currently being leveraged toward mitigating or preventing disease. That's definitely a recurring theme in each plenary session. It does set the meeting apart. There are wonderful basic neuroscience meetings throughout the year, but the ANA has a much stronger mission to understand potential for and realization of neurological therapeutics.

Q. What are some of the scientific highlights that attendees can anticipate?

The Presidential Symposium on Lewy body dementia (LBD) will be a highlight, not only for the science but because Susan Schneider Williams, widow of the actor Robin Williams who had LBD, will participate on the panel and share her experience.

Advances in cell-based therapeutics is also highly anticipated. In our SPAC committee meetings, it got a 100% interest rating among the members.

The session on disease-modifying therapies in traumatic brain injury is also of broad interest. One of the discussions will center on the remote effects of brain trauma – the risk that may be borne years out and what might be done to interfere with that process. I believe there will also be discussion on the science behind how to manage pediatric brain injury – and when it is safe to return to sports after injury.

This year the pre-meeting symposium presents the latest advances in viral vectors for delivery of gene therapy. This will be particularly interesting because one of the principal issues in gene therapy is the mode of delivery – getting to sufficient numbers of the cells that need to be impacted and to do so in a safe way. Indeed this year saw the first viral-vector delivered gene therapy to receive FDA approval, indicating this form of therapy is about to realize its clinical potential.

The role of inflammation in neurological disorders has been of interest for some time and it has developed a rather bad reputation. We're gaining a richer appreciation of the helpful roles of inflammation, so this session should provide an interesting point/counterpoint, offering more nuanced, balanced approach to therapies that target this process.

And in the plenary session on the vascular contributions to dementia, the message is that the blood vessels in the brain are relevant for more than just their role in strokes. It has become clear that the brain's vascular system has a great influence on the pathogenesis of dementias, including the Alzheimer type. Hypertension is actually a risk factor for cognitive decline and the beta amyloid pathway so closely linked to Alzheimer's has an impact on vascular as well as neuronal function. This realization has brought interesting insights into early indicators of cognitive decline and new potential targets for treatment and prevention of dementia.

Q. What's unique about the ANA meeting?

While each of the six plenary sessions has a theme, there are presentations on several different disorders within that theme, so at the end of the session you have a much richer overview of what is possible and what's coming.

It's rare that you get a national conference with such a concentration of academic neurologists and physician scientists thinking about the causes and treatment of neurological disease. We're usually diluted and scattered, so the ANA meeting is particularly exciting for an investigator, physician or not, who is interested in the translation of basic science to clinical applications.

I think it's going to be an incredibly exciting meeting with important information across multiple age groups and neurological conditions, which often have certain common features that will provide insights applicable to many subfields.

###

For a preview of the ANA 143rd Annual Meeting, please click here.
To register for the meeting, please click here.