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ALL BRAINS ON DECK TO FIGHT NEURODEGENERATIVE DISEASES

A conversation with **ROBERTA DIAZ BRINTON**, Ph.D. director of The Center for Innovation in Brain Science



Neurodegenerative diseases affect more than 62 million people worldwide. The Center for Innovation in Brain Science (CIBS) at the University of Arizona merges translational and clinical research to advance the creation of precision therapeutics. Tight integration with faculty operated biotech startups and the development industry partnerships ensures the translation and delivery of innovative precision therapeutics. A testament to the model's success is a recent \$37.5 million grant from the National Institutes of Health for a phase 2 clinical trial testing a potential regenerative therapeutic for Alzheimer's Disease.

What is the main challenge that CIBS addresses?

There is not a single cure for a single neurodegenerative disease. Our focus is on the four major age-associated neurodegenerative diseases: Alzheimer's, Parkinson's, Multiple sclerosis and ALS (Amyotrophic Lateral Sclerosis). Enormous intellectual and financial resources have been brought to bear on these; yet cures remain elusive.

We must think differently about the origin and trajectory of these diseases. A common factor in each is advanced age which is the most significant factor for these diseases. Our approach focuses on understanding that ageing is a dynamic process, comprising multiple distinct transition states and that these diseases involve multiple systems of biology.

How do you achieve this goal?

CIBS is an integrated translational ecosystem focused on the four major age-associated neurodegenerative diseases. The expertise of our faculty and research staff spans the spectrum of translational innovation: from basic disease biology, brain bioenergetics, computational systems biology, bioinformatics, structural biology, medicinal chemistry, translational animal model development, to clinical trial design. Collaborations within the centre take the form

of dedicated design teams, comprising faculty from different disciplines focused on finding and validating new targets and developing therapeutics.

At CIBS, once a promising candidate has been identified, a team of regulatory and clinical experts ensures that all Investigational New Drug (IND)-enabling requirements are met to ensure it moves through the pipeline.

Why is this approach unique?

CIBS is a hybrid between academic research and biotech translational knowhow. We have an 'all brains on deck' culture where we share intelligence and resources. Because we are all located together, we can discuss issues, answer questions and advance our research in real time. Part of our success is that we are nimble and can integrate enabling innovations across our research endeavors. This is one of the most important aspects of our success, we can test innovation in one disease category, and once proven successful we can seamlessly expand that innovation across multiple neurodegenerative diseases. We understand that to achieve our goal of delivering precision therapeutics we will have to both develop startups and partner with existing companies. In fact, most of our investigators have launched one or more startups.

What advancements has CIBS made recently?

A major effort is the development of the first regenerative therapeutic for Alzheimer's disease. Our research into the regenerative potential of the neurosteroid allopregnanolone has transitioned from mechanistic discovery science to IND-enabling translational research, and then to a phase 1 clinical trial in people with Alzheimer's. Based on outcomes from our mechanistic, translational and phase 1 clinical trial research, we are advancing a precision medicine approach in our National Institute on Ageing funded Phase 2 clinical trial of - allopregnanolone for patients that carry the APOE4 genetic risk factor.

How does this advancement in Alzheimer's treatment apply to other neurodegenerative diseases?

Our regenerative therapeutic for Alzheimer's is one example of the therapeutics being developed by CIBS investigators, and its success is a huge endorsement of our model. Our portfolio includes novel therapeutics that target pathways for regeneration, protein aggregation, neuroinflammation and brain bioenergetics. I am very excited that since launching in 2016 we have been able to identify therapeutics for Alzheimer's, Parkinson's, Multiple sclerosis, and ALS, whose stage in our pipeline ranges from early

development to clinical trials. The University of Arizona has an excellent intellectual property office and we have been able to file patents for our discoveries.

What impact does your work have on patients?

We are living longer, but not necessarily better. Most neurodegenerative diseases are chronic conditions. They are an unrelenting physical and financial challenge for the person with the disease as well as their family, community and nation. Curing one person of one of these diseases has a broad ripple effect; when a person is cured of a neurodegenerative disease, that person is freed of the disease, as are their family and community.

What's next?

These diseases are personal, and our therapeutics should be too, while we have a promising portfolio, our pipeline of therapeutics will expand to advance precision medicine for neurodegenerative disease. Our approach has been successful. Going forward, we will continue to innovate and expand our therapeutic horizon to bring innovations in brain science of the future to those who need a cure today.



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The Center for Innovation in Brain Science (CIBS) is an all brains on deck research environment designed for integrated collaborative research through innovative team science.

Make **A Difference**

CIBS is recruiting postdoctoral fellows with expertise in any of the following areas: systems biology, bioinformatics, immunology, synthetic chemistry, drug design, stem cell technology, deep/machine learning and computational systems biology.

Be a part of a rich training environment and a diverse, mission-driven team focused on discovery and creating precision cures for Alzheimer's, ALS, MS and Parkinson's.

Why **Work At CIBS?**

Challenging, impactful work. Highly competitive benefits package.

Learn more at: bit.ly/cibscareers



