

## Translating Scientific Discoveries to Impact the Atlanta Community and Beyond

### MISSION

ACTSI, led by Emory University and partners, Morehouse School of Medicine and Georgia Institute of Technology, is a catalyst for moving biomedical discovery into clinical applications. ACTSI also educates new clinical and translational investigators, provides pilot funding and scientific review and supports areas critical to successful clinical and translational research such as bioinformatics, biostatistics, ethics and regulatory affairs, community dissemination, research technologies and access to over 30 clinical research sites across Atlanta.

Since inception in 2007, ACTSI has supported 843 investigators who are conducting 234 grants totaling \$186,208,944 resulting in 961 publications and 33 patents, and trained over 75 clinical research scholars. Visit [www.ACTSI.org](http://www.ACTSI.org) to learn more.

### THE REMOTOSCOPE

Wilbur Lam, MD, PhD (Emory/GT Biomedical Engineering Program) and Erik Douglas (CEO of Cellscope) have introduced the Remotoscope.

- A clip-on attachment and software app that turns an iPhone into a digital otoscope

Ear infections are the most common diagnosis for preschoolers, resulting in more than 30 million office visits/year in the U.S. and thousands of prescriptions for antibiotics.

The Remotoscope:

- reduces emergency room visits for kids who get ear infections frequently
- reduces unnecessary antibiotic use

Other Funding: FDA, Atlanta Pediatric Device Consortium



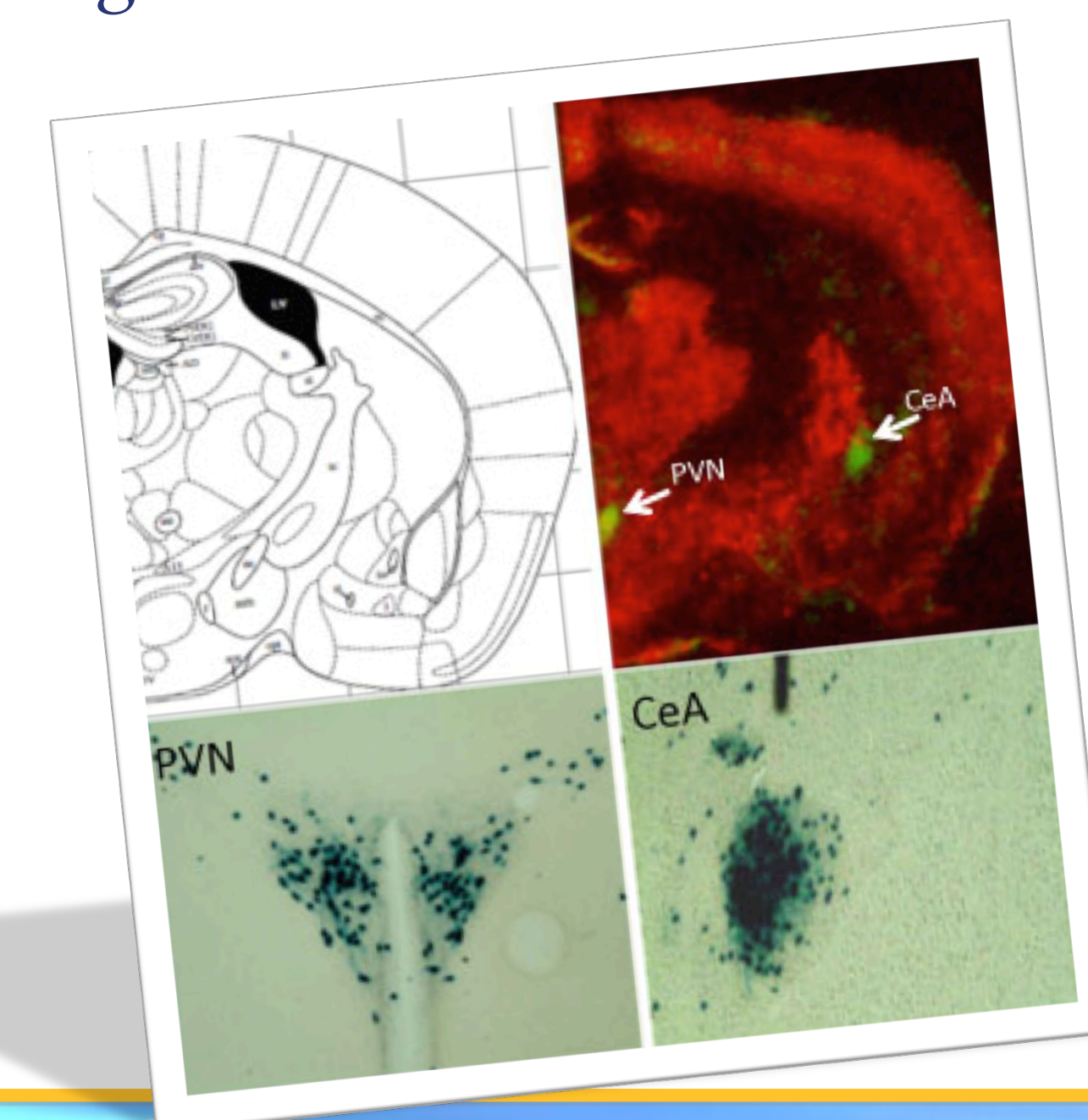
### KEY BIOLOGICAL PATHWAY LINKED TO PTSD

Kerry Ressler, MD, PhD (Psychiatry, Emory) and collaborators discovered a key biological pathway, PACAP (pituitary adenylate cyclase-activating polypeptide), linked to PTSD.

- Genetic and Trauma-Related Risk Factors for Posttraumatic Stress Disorder (PTSD)
- Prospective Determination of Psychobiological Risk Factors for Posttraumatic Stress Disorder
- Cortisol Suppression and Startle Responses in PTSD

Publication: Post-traumatic Stress Disorder is Associated with PACAP and the PAC1 Receptor, *Nature*, 2011, PMC3046811

Other Funding: NIMH R01MH094757  
NIMH R01MH1113535



### HEART FAILURE CLINICAL TRIALS NETWORK

Javed Butler, MD, MPH (Cardiology, Emory) leads the Heart Failure Clinical Trials Network, a cooperative network of advanced heart failure clinical and research centers charged with accelerating innovative research and developing strategies to diagnose, manage and treat all forms of heart failure.

The NHLBI-funded network has nine members and is working toward a common goal of finding better treatment options for patients with heart failure, through projects like:

- The Atlanta Cardiomyopathy Consortium (TACC)

Publication: Validation of the Health ABC Heart Failure Model for Incident Heart Failure Risk Prediction: The Cardiovascular Health Study, *Circulation*, 2010. PMC3285297

Other Funding: NHLBI U10HL110302

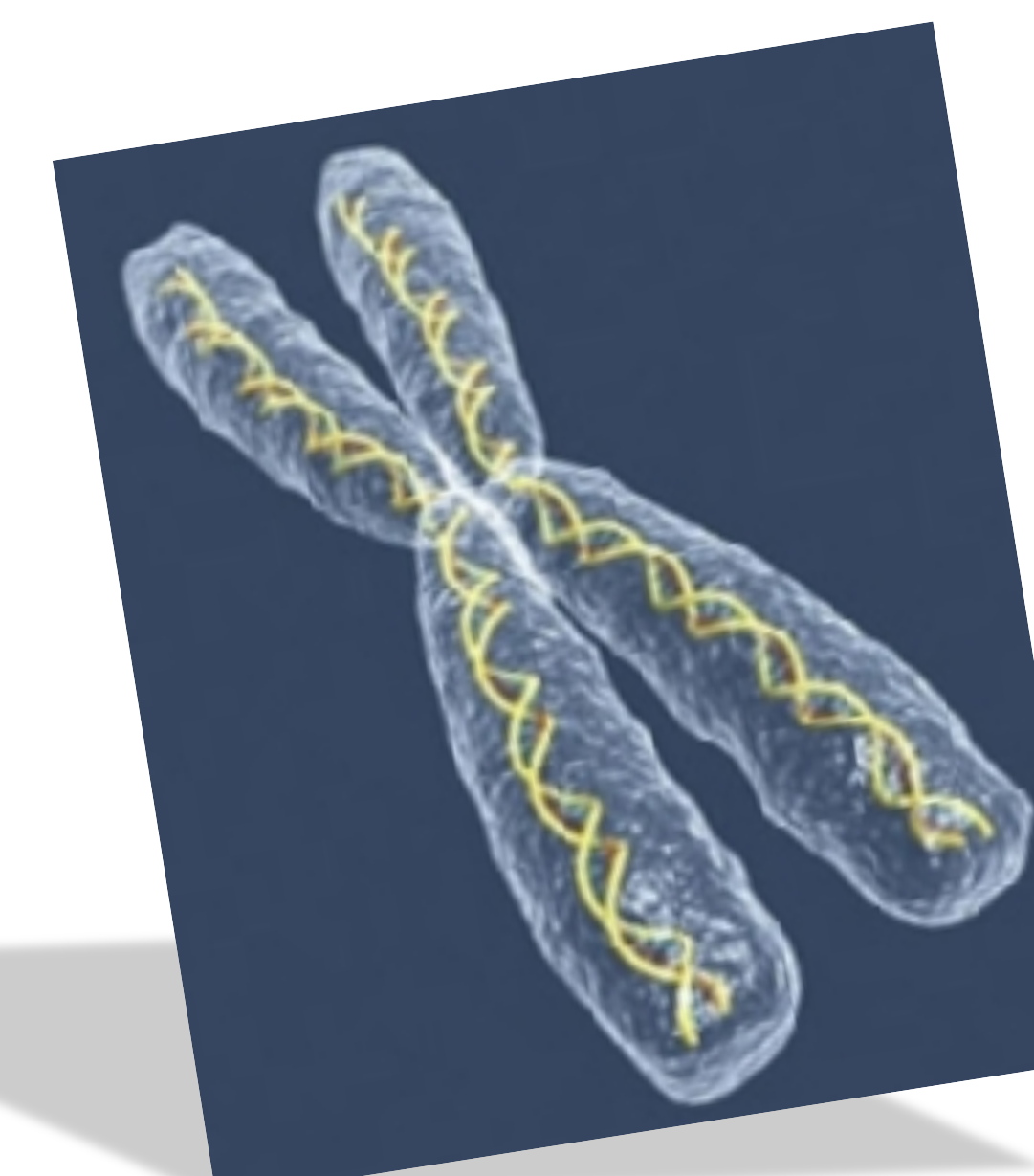


### MUTATIONS IN AUTISM SUSCEPTIBILITY GENE INCREASE RISK IN BOYS

Michael Zwick, PhD (Human Genetics, Emory) director of the ACTSI-supported Emory genomics core used RainDance Technologies RDT 1000 instrument for multiplex PCR to enhance targeted sequencing capabilities and:

- Identified five rare mutations in a single gene, AFF2, that appear to increase the chances that a boy will develop an autism spectrum disorder.
- Mutations in the AFF2 gene, and other genes on the X chromosome, may help explain why autism spectrum disorders affect four times as many boys as girls.

Publication: Excess Variants in AFF2 Detected by Massively Parallel Sequencing of Males with Autism Spectrum Disorder, *Human Molecular Genetics*, 2012, PMC3492087  
Other Funding: NIMH MH076439



### INNOVATIVE BONE MARROW THERAPY TO TREAT CROHN'S DISEASE

This Phase I investigator-initiated, dose escalation study, led by Subra Kugathasan, MD (Pediatrics/Human Genetics, Emory) and Jacques Galipeau, MD (Hem/Onc/ Pediatrics, Emory), addresses the safety and tolerability of autologous bone marrow derived Mesenchymal Stromal Cells (MSC) in patients with Crohn's Disease.

- MSCs have been shown to impact autoimmune conditions such as Graft versus Host Disease (GvHD) and inflammatory bowel disease (IBD).

Infusions for this first-in-human trial are underway at the ACTSI Pediatric Research Center, Children's Health Care of Atlanta, Egleston Hospital. To date four controls are enrolled and one active participant, an 18 year old female. The first patient infusion was done in March 2013.

