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‘One Big Community’

UAMS/ACRI Allergy Study Underscores Clinical Trials’ Importance

Amy Scurlock, M.D., and Stacie Jones, M.D. (photo courtesy of Arkansas Children’s)

A recently approved treatment for multiple food allergies highlights the significance of clinical trials for Arkansans as well as a culture of collaboration that benefits clinical trials, and often study participants, across UAMS and Arkansas Children’s Hospital and Research Institute.

The federally funded multi-site study that included UAMS allergy researchers based at Arkansas Children’s found that the drug omalizumab substantially reduced potentially life-threatening reactions in patients with peanut and other common food allergies. The Food and Drug Administration (FDA) approved the drug based on the clinical trial’s initial findings, which were published Feb. 25 in the *New England Journal of Medicine.*

Stacie Jones, M.D., is the UAMS/Arkansas Children’s site principal investigator on the study. She and Amy Scurlock, M.D., are co-authors on the NEJM report.

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Dear Colleagues,

In this issue of The TRIbune, we highlight the outstanding work of our UAMS colleagues and CTSA participating partners at Arkansas Children’s Hospital and Research Institute who are serving Arkansans through their clinical trials leadership.

Stacie Jones, M.D., is the UAMS principal investigator on the multi-site study finding that omalizumab substantially reduced potentially life-threatening reactions in children and adults with peanut and other common food allergies. The Food and Drug Administration approved the drug based on the clinical trial’s initial findings, published in the *New England Journal of Medicine.*

The clinical trials led by Dr. Jones over her career are models for all researchers. She is a great example of how successful clinical trialists, funded over the course of their careers by NIH networks, are doing tremendous work to change how we care for patients. Food allergy patients in Arkansas are benefiting because of her efforts.

As with the omalizumab study, long before the FDA approves a drug, Arkansans with serious conditions may benefit from treatment as clinical trial participants. In that way, clinical trials are “filling in gaps” in therapeutics.

Dr. Jones’ approach has always been built on a spirit of teamwork. This includes her role as a TRI liaison to the national CTSA Trial Innovation Network, which is helping bring additional clinical trials to researchers at Arkansas Children’s Research Institute.

Sincerely,

Laura James, M.D.
Director, TRI
Associate Vice Chancellor for Clinical and Translational Research, UAMS

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Both are professors in the College of Medicine Department of Pediatrics, Division of Allergy and Immunology, and they are based at Arkansas Children's.

'Tremendous Impact'

Led by Johns Hopkins Children's Center, the multi-site Phase 3 OUtMATCH study was funded by the NIH National Institute of Allergy and Infectious Diseases with support from the NIH National Center for Advancing Translational Sciences (NCATS), which also funds TRI.

"We estimate that 40% of the millions of people in the United States who cope with food allergies are allergic to multiple foods," said Jones, who led the study at the Arkansas Children's Research Institute (ACRI), one of 10 sites in the U.S. conducting the OUtMATCH study through the Consortium for Food Allergy Research. "This treatment demonstrated a tremendous impact for the most study participants by increasing the amount of allergen required to cause a reaction. This finding translates to increased protection for patients providing a way for them to live with less fear of an allergic reaction."

Omalizumab, an injectable drug, follows Palforzia, a peanut oral immunotherapy approved in 2020, as the second FDA-approved food allergy drug that was also studied by Jones and Scurlock. Omalizumab was previously FDA-approved and prescribed at Arkansas Children's for asthma and chronic hives. The treatment is now available for qualifying food allergy patients.

Access to Emerging Drugs

"There is a huge unmet need, and we want new drugs for the conditions that we treat and the life-threatening anaphylaxis that children and adults face every day," said Jones, who led her first UAMS/Arkansas Children's clinical trial in 1994 and her first food allergy clinical trial in 2000. "Clinical trials such as these give people in Arkansas and our region early access to new therapies years ahead of when that drug becomes available to patients. It helps fill some of the gaps in treatment by providing these emerging therapies to patients early."

Scurlock echoed the excitement for the recent breakthroughs and reflected on the long journey of discovery. "I started my career here almost 20 years ago and up until very recently there were no FDA-approved products for the treatment of food allergies," she said.

The Real Heroes

Jones and Scurlock emphasized the importance of the Arkansas Children's Food Allergy research team who provide the day-to-day care of study participants and are key to the program's success. They also emphasize that the real heroes of their research success are the study participants and families who participate.

"They give us years of their lives and entrust us to create and maintain a safe environment for their children," Jones said.

Input from families also helps drive researchers' priorities, they said.

"When we first started doing this work in food allergy, we wanted a 'cure,' or wanted to make it so patients could tolerate the peanut or the food and put it back in their diet," Scurlock said. "But what many parents and families want is to have assurances that their child can attend school or activities away from home with protection from an allergic reaction."

"Their engagement tells us so much more than just what therapy is going to work," Jones said. "It helps us understand their preferences, fears, risk perception, and behavior related to their food allergy. Our study participants – kids and adults – contribute so much more than just getting that drug on the market."

TRI Connections

Jones, serving since 2019 as a TRI liaison for NCATS' Trial Innovation Network (TIN), exemplifies the collaborative ethos and emphasis on bringing nationally relevant clinical trials to UAMS and ACRI. The efforts are yielding additional clinical trials and potential treatments to Arkansas patients.

Jones' affiliation with TRI and the TIN regularly brings new clinical research opportunities to pediatric investigators, with three TIN-initiated clinical studies now open at Arkansas Children's.

The Arkansas Children's Food Allergy Program has 23 active clinical research studies in children and adults with food allergy and related disorders. Meanwhile, there are more than 150 ongoing clinical studies led by UAMS faculty with TRI support, such as funding, resources, or services. While most of the studies are UAMS- or ACRI-based, a few, primarily in neonatology, involve participants at both UAMS and Arkansas Children's.

The strong ties between TRI and Arkansas Children's includes robust participation by pediatrics faculty in numerous TRI-sponsored training and funding programs.

For example, Jones, Scurlock and other UAMS/Arkansas Children's faculty serve as mentors for medical students selected to participate in TRI's summer Translational Research Innovations and Partners (TRIP) program. The program is a partnership with the College of Medicine Honors in Research program.

"The way I see it is, although we are pretty kid-centric at Arkansas Children's, we are one big community with UAMS, and it takes all of us working together to make a meaningful difference for our patients," Jones said.

Translational Research vs. Translational Science What's the Difference?

The terms translational research and translational science have been used interchangeably for more than a decade, but that's changing across Clinical and Translational Science Awards (CTSA)-funded institutions, including TRI.

Driving the transformation is the NIH National Center for Advancing Translational Sciences (NCATS), which funds the CTSA program. Beginning in July of 2024, TRI supported awardees and trainees will need to explain how their research addresses both translational research and translational science. **Translational research** addresses overcoming a barrier to propel a specific research project forward to develop new treatments or treatment approaches for patients. **Translational science** takes a step back from the specific research project to ask broader questions such as a) how can the research approach support future research projects or be used by other researchers? or b) how can barriers to research be mitigated to support the broader research community and to support future research? Barriers may be scientific or administrative.

Translational science is the "science of translational research," said TRI Director Laura James, M.D. The ultimate goal of translational science is to identify "best practice approaches" for translational research that have broad application for numerous researchers and research disciplines. One recent example of translational science is the wide-scale use of electronic health records to better understand the impact of COVID-19 on specific patient groups. NCATS created the **NC3 data enclave** – a national data infrastructure based on electronic health records from over 75 institutions - to better understand COVID-19.

A recorded video presentation on translational research and translational science is now available. The video is a condensed version of a recent TRI seminar led by J. Rob Singleton, M.D., director of the University of Utah's Clinical Research Unit at the Clinical and Translational Science Institute.

View the presentation here or use the QR code.

Scan Here

Tachinardi U et al. Privacy-preserving record linkage across disparate institutions and datasets to enable a learning health system: The national COVID cohort collaborative (N3C) experience. Learn Health Syst 2024 Jan ; 8(1): e10404.

TRI Study of the Month

Principal Investigator: Nishank Jain, M.D., associate professor, UAMS College of Medicine, Department of Internal Medicine, Division of Nephrology; TRI KL2 Mentored Research Career Development Program Scholar

Summary: An investigator-initiated study analyzing blood samples of adults with chronic kidney disease (CKD), adults with kidney transplants, and healthy adults to understand the interactions between platelets and leukocytes as possible drivers of inflammation in CKD.

Significance: CKD patients have abnormal inflammation associated with higher risks for heart attack and stroke. Having established that platelets are a primary cause, Jain hopes to identify the abnormal inflammatory pathways in CKD patients so that therapeutics may be found to inhibit platelets and reduce patients' inflammatory burden.

TRI Services: ARresearch participant registry

Funding Agency: NIH National Center for Advancing Translational Sciences

Learn more about the participant registry at TRI.uams.edu and ARresearch.org.

Nishank Jain, M.D., (center) holds an iPad displaying the ARresearch website, where more than 9,400 Arkansans have registered as potential research participants. Michelle White, RN (left), helped successfully recruit participants to Jain's study using the ARresearch registry, and Pam Christie manages the registry for TRI.

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4301 W. Markham St., #577
Little Rock, AR 72205-7199

Journal Devotes Issue to Clinical Trials Project Led by TRI's Laura James, M.D.

TRI Director Laura James, M.D., concluded her tenure as co-chair of the national Clinical and Translational Sciences Award (CTSA) Steering Committee in December with an effort aimed at making clinical trials more informative and of higher quality. Her work, and that of other CTSA leaders, appears in a themed issue of the *Journal of Clinical and Translational Science (JCTS)*, published in February.

James led a team of five guest editors from CTSA institutions across the United States and led the journal issue's editorial, "Scientia Pro Bono Humani Generis: Science for the Benefit of Humanity," which introduces readers to the emphasis of the February issue.

The work was inspired by a 2019 paper in JAMA entitled, "Harms from Uninformative Trials." The JAMA authors defined an uninformative trial as one lacking in meaning by the patient, clinician, researcher, or policymaker.

In the JCTS editorial, James and her co-authors acknowledge the problems associated with uninformative clinical trials, writing, "Academic health organizations, funding agencies, and clinical trialists have been challenged to optimize clinical trial informativeness, and quality issues continue to plague the development and conduct of clinical trials."

Multiple potential solutions are offered in the journal's manuscripts, which highlight innovations for enhancing the informativeness and quality of clinical trials.

One innovative example for improving clinical trial efficiency is the use of adaptive trials, James said. Adaptive trials use prespecified rules to modify the course of a trial and to optimize it based on the incoming results.

"Adaptive trial designs are a new approach to clinical trials that are moving us away from traditional double-blind placebo-controlled trials," James said.

Infrastructure, training, participant recruitment and other factors are addressed in the report.

"We looked at multiple aspects of uninformative clinical trials in this issue, so we addressed common problems at the institutional as well as the study level," James said. "We're asking, what are the academic health organizations doing to ensure that their trials are of the highest quality, and are they really going to have an impact on human health?"

She added: "As stewards of public funds that support the development of clinical trials, it is critical that we optimize clinical trial designs so that we create trials that move us forward in improving the health of individuals and communities."

View the JCTS publication here, or use the QR code. Scan Here

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Editor *David Robinson*

Designer *Amber Fiser*

TRI Director *Laura James, M.D.*