Patients Are Eager for Trials But Aren’t Getting the Word, Survey Finds
By Bill Myers

Patients are eager to participate in clinical trials, but they’re having trouble finding the information they need — not just on social media, but from their own doctors, a new survey suggests.

Many respondents said they’d be interested in joining trials — nearly three-fifths said they would be willing to share genetic information or their health records as part of a trial — and one in five said they had proactively asked their doctors about trials, but only 15 percent had ever enrolled in one. Fewer than one in five said their doctor had suggested trials for them.

The survey was conducted by Inspire, a healthcare-focused social media platform, which surveyed nearly 9,500 of its more than 1 million patient and caregiver members about a range of issues, including awareness of clinical trials.

There also seems to be something of a gender gap in patient readiness for trials. Nearly one-quarter of men said their doctor had suggested a trial for their diseases, but fewer than one-fifth of women received similar advice.

This is the fourth year Inspire has surveyed patients and caregivers about their treatment. The ongoing lesson, Inspire Research Manager Hannah Watson Eccard says, is that people “are more than recipients of medical care. They are active partners who work with healthcare professionals to make medical decisions and find the best option for them.”

see Eager for Trials on page 4

Drug Industry Comments on FDA’s Real-World Evidence Program
By Bill Myers

Drug sponsors are eager for the FDA to embrace real-world evidence (RWE), but several companies are asking the agency to clarify how it can be used in clinical trials.

Late last year, the FDA invited public comments on a plan for its real-world evidence framework, and it received 30 overall, nine from drugmakers.

One of the major threads in the comments was about data: specifically connectivity, gaps and capture. The framework “does not provide clarity regarding the kinds of data standards required, who is responsible for developing data standards or the process by which FDA will assess the acceptability of those standards,” Janssen wrote. “Will FDA require different data standards for different data sources (e.g., health insurance claims vs. electronic health records) or for different types of regulatory decisions?”

Another thread was about evidentiary standards and adequate scientific evidence. “There is not enough detail for sponsors to know whether their evidence will satisfy these criteria. We realize that the practice of using RWE for regulatory decision-making outside of addressing safety questions is still in its infancy,” Janssen added.

Some sponsors also latched on to language that suggested the agency might be open to using observational studies that “replicate” randomized trial results and were worried that the word is a little too
FDA Promises Flexibility for Stem Cell Trials
Regulators are promising “flexibility” in clinical trial design for stem cell therapies in rare diseases.

In a final guidance issued last week, the FDA says it “recognizes that, for regenerative medicine therapies for rare diseases, certain aspects of drug development that are feasible for common diseases may not be feasible, and that development challenges can be greater with increasing rarity of the disease.”

“Innovative trial designs, such as trials that compare several different investigational agents to each other and a common control, may be particularly useful in studies of regenerative medicine therapies to treat rare diseases,” the guidance says.

CBER might also be open to historical controls data obtained from natural histories, and regulators might be willing to consider a trial that — unlike the traditional, multi-site study — uses multiple sites that are sharing data on stem cell therapies in a combination to support a BLA.

Sponsors are encouraged “to obtain input from the affected patient communities” when they’re coming up with endpoints for stem cell trials.

The FDA also is promising to be flexible about fast-tracking some potential stem cell treatments. The agency will want clinical data to justify a fast-track application, but the data doesn’t necessarily have to come from prospective clinical trials.

“In some cases, clinical evidence obtained from clinical investigations with appropriately chosen historical controls may provide sufficient preliminary clinical evidence of the potential to address an unmet medical need,” the agency says. “In other cases, preliminary clinical evidence could come from well-designed retrospective studies or clinical case series that provide data systematically collected by treating physicians.”


FDA Issues Draft Guidance on Evaluation of Devices Used With RMATs
New guidance issued by the FDA on Friday outlines the conditions under which clinical evidence will be required in a marketing authorization application for a device used in recovery, isolation or delivery of regenerative medicine advanced therapies (RMATs).

In cases in which sponsors submit separate applications for the RMAT and the device, they have the option to streamline or simplify the regulatory process. “For example, when appropriate and legally permissible, clinical or performance data generated in association with studies of one product may be submitted to support certain aspects of the approval or clearance of other, related product applications,” the guidance states. In other cases, it may be possible for one regulatory submission to refer back to existing performance data from another submission when available.

In cases where a device may be intended for use with only one particular type of cell-based RMAT or with more than one type, it may be necessary to repeat the testing process to assess interactions between the two components for each combination. Sponsors should provide detailed and specific justifications for the applicability of the data and provide sufficient compatibility data ahead of clinical trials.

Read the draft guidance here: https://bit.ly/2DNzzaC.

FDA Gives Priority Review to Keytruda
Merck has won priority review for its blockbuster anti-cancer drug Keytruda after promising data from a pivotal Phase III trial, the company announced last week.

The FDA granted priority review for Merck’s supplemental BLA for its blockbuster immunotherapy Keytruda (pembrolizumab) for head and neck cancers. It’s already approved as a second-line treatment for head and neck cancer and several other forms of cancer. The drug has shown “significant improvement” for head-or-neck cancer patients’ overall survival in a Phase III trial, the company said.

The FDA committed to completing the priority review no later than June 10.

Novartis’ Fevipiprant Makes Asthmatic Muscles Smooth, Researchers Find
Novartis’ anti-asthma medication Fevipiprant appears to be the first anti-asthma drug ever to smooth out the muscles lining patients’ airways and may be the first new asthma drug to be approved in 20 years.

Fevipiprant is already in a Phase III trial involving nearly 1,600 patients, scheduled to wrap up by 2022. Researchers from England and Canada, though, crunched the data behind the drug’s last trial, a Phase IIa trial that involved 61 patients from 2012-13.

They found that, like most asthma medicines, Fevipiprant helped ease inflammation in the lungs of its patients, but it also reduced...
the amount of muscle in the lining of the lungs. An increase in smooth muscle is the strongest predictor of restricted airflow.

**Texas Women’s Clinic Signs Up for Trials Network**
The Corpus Christi Women’s Clinic has agreed to join Elligo Health Research’s clinical trials network, the clinic announced last week.

Elligo, a patient recruitment company, offers Corpus Christi Women’s Clinic “the infrastructure... necessary to conduct research in its office, effectively keeping patients closer to home and under the care of their trusted physician,” the clinic said in a news release.

Under the agreement, several of the clinic’s doctors have agreed to offer themselves as principle investigators in clinical trials.

**Charles River Spends $510 Million for Citoxlab**
Charles River Laboratories has agreed to acquire private safety watchdog Citoxlab for nearly $510 million, the companies announced last week.

The acquisition gives Charles River — which claims to have participated in 85 percent of successful drug trials last year — a larger footprint in Europe. Citoxlab offers an array of early-stage services, from specialized toxicology to genomics.

Charles River officials are also hopeful that the deal will expand the CRO’s small- and mid-sized biotech client base. Small- and mid-sized sponsors have been Charles River’s fastest growing customer segment.

The acquisition is expected to close by the second quarter of this year.
Eager for Trials
continued from page 1

For some patient advocates, the Inspire survey is wake-up call to the trials industry. “We need to do a better job of initiating conversations about new treatment options — especially clinical trials,” says Dana Dornsife, founder of the Lazarex Cancer Foundation, a California nonprofit that helps patients overcome obstacles to trial enrollment.

Dornsife says she and her colleagues “receive calls daily from cancer patients for whom standard care isn’t working and can’t find the information they need as they investigate clinical trial options.” The group is in the midst of a project it calls IMPACT, a three-year pilot program aimed at improving enrollment in trials.

“The system is fragmented and flawed and leaves many patients out,” she says. “And if a patient does find a clinical trial for which they’re eligible, they’re often blindsided, in many cases, by insurmountable travel costs just to get to the clinical trial.”


### Participation in Clinical Trials

- **15%** have participated in clinical trials.
- **19%** have had a doctor suggest a clinical trial.
- **21%** have proactively asked their doctor about a clinical trial.
- **10%** both had a doctor suggest a clinical trial and proactively asked their doctors about a clinical trial.

**Source: Inspire**

### Interest in Participating in Genetic Research

- **33%** of patients have heard of precision medicine.
- **57%** of patients would be willing to share both DNA and health records with a hypothetical clinical research project.
- **22%** have already had their DNA tested, and an additional **60%** are interested in having their DNA tested.
- **17%** would be willing to share either their DNA or their health records, but not both.

**Source: Inspire**
“There is not enough detail for sponsors to know whether their evidence will satisfy these criteria.”

—Janssen Research & Development, LLC

Novartis also suggested that sponsors should be more prominent in the demonstration projects that the FDA is developing. The company commented on the “clear lack of industry (i.e., drug sponsors) participation” in those projects.

Regeneron suggested that the FDA “provide some discussion points to assist sponsors in how best to apply practices or lessons learned from these programs to planned or future studies to assist in regulatory decision-making, particularly in areas of high unmet medical need.”

Novo Nordisk said it would like to see examples of adaptive or Bayesian trial designs, which the FDA has said might help qualify biomarkers for trials. The agency should also be open to using preclinical data for biomarkers, Novo said.

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## Drug & Device Pipeline News

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<tr>
<th>Company</th>
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<th>Medical Condition</th>
<th>Status</th>
<th>Sponsor Contact</th>
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<tbody>
<tr>
<td>ADC Therapeutics</td>
<td>ADCT-402 (loncastuximab tesirine) plus AstraZeneca's IMFINZI (durvalumab)</td>
<td>advanced diffuse large B-cell lymphoma (DLBCL), mantle cell lymphoma (MCL) or follicular lymphoma (FL)</td>
<td>Phase I trial initiated enrolling 75 subjects</td>
<td>adctherapeutics.com</td>
</tr>
<tr>
<td>Exelixis, Inc.</td>
<td>XL092</td>
<td>cancer</td>
<td>Phase I trial initiated</td>
<td>exelixis.com</td>
</tr>
<tr>
<td>ReGenTree, LLC and Ora, Inc.</td>
<td>RGN-259</td>
<td>dry eye syndrome</td>
<td>Phase III trial initiated enrolling 700 subjects</td>
<td>regentreellic.com, oraclinical.com</td>
</tr>
<tr>
<td>LASEROPTEK Co., Ltd.</td>
<td>PicoLO picosecond Nd:YAG laser</td>
<td>dermatology and general and plastic surgery</td>
<td>510(k) clearance granted by the FDA</td>
<td>laseroptek.com</td>
</tr>
<tr>
<td>Grifols, S.A.</td>
<td>Proceix Babesia assay</td>
<td>blood screening</td>
<td>510(k) clearance granted by the FDA</td>
<td>grifols.com</td>
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<tr>
<td>Finch Therapeutics Group, Inc.</td>
<td>CP101</td>
<td>recurrent <em>Clostridium difficile</em> (<em>C. difficile</em>) infection</td>
<td>Breakthrough Therapy designation granted by the FDA</td>
<td>finchtherapeutics.com</td>
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<tr>
<td>RDD Pharma, Inc.</td>
<td>RDD-0315</td>
<td>fecal incontinence in spinal cord injury</td>
<td>Fast Track designation granted by the FDA</td>
<td>rddpharma.com</td>
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<tr>
<td>Neurotech Pharmaceuticals, Inc.</td>
<td>NT-501 (Renexus)</td>
<td>macular telangiectasia type 2 (MacTel)</td>
<td>Fast Track designation granted by the FDA</td>
<td>neurotechusa.com</td>
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<tr>
<td>IntraBio, Inc.</td>
<td>IB1001</td>
<td>GM2 Gangliosidosis (Tay-Sachs and Sandhoff Disease)</td>
<td>IND approval granted by the FDA</td>
<td>intrabio.com</td>
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<tr>
<td>Pulmatrix, Inc.</td>
<td>Pulmazole (PUR1900)</td>
<td>allergic bronchopulmonary aspergillosis (ABPA) in patients with asthma</td>
<td>IND approval granted by the FDA</td>
<td>pulmatrix.com</td>
</tr>
<tr>
<td>Precigen, Inc.</td>
<td>PRGN-3005</td>
<td>advanced-stage platinum-resistant ovarian cancer</td>
<td>IND approval granted by the FDA</td>
<td>precigen.com</td>
</tr>
<tr>
<td>Janssen Pharmaceutical Companies of Johnson &amp; Johnson</td>
<td>Darzalex (daratumumab) split-dosing regimen</td>
<td>multiple myeloma</td>
<td>BLA approval granted by the FDA</td>
<td>janssen.com</td>
</tr>
<tr>
<td>Sanofi</td>
<td>Cablivi (caplacizumab-yhdp) in combination with plasma exchange and immunosuppression</td>
<td>acquired thrombotic thrombocytopenic purpura (aTTP) in adults</td>
<td>BLA approval granted by the FDA</td>
<td>sanofi.com</td>
</tr>
<tr>
<td>Medtronic</td>
<td></td>
<td>expanded indication for Pipeline Flex embolization device</td>
<td>Approval granted by the FDA</td>
<td>medtronic.com</td>
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<tr>
<td>Orthofix Medical, Inc.</td>
<td>M6-C artificial cervical disc</td>
<td>cervical disc degeneration</td>
<td>Approval granted by the FDA</td>
<td>orthofix.com</td>
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For news on trial results, FDA approvals and drugs in development, join the LinkedIn Drug Research Updates group!
Upcoming Event Highlights

Conferences

MARCH 26-27, 2019
Conducting Advanced Root Cause Analysis and CAPA Investigations
Raleigh, NC

MARCH 27-28, 2019
ICH GCP E6 R2 Meeting CRO-Vendor Oversight Requirements
Raleigh, NC

APRIL 8-12, 2019
FDA Compliance Boot Camp 2019
Frederick, MD

APRIL 15-18, 2019
18th Annual Design of Medical Devices Conference
Minneapolis, MN

Webinars

FEBRUARY 27, 2019
FDA Medical Device Compliance and Enforcement: Lessons from Last Year and Your Game Plan for This Year

- What areas the FDA targeted in inspections in 2018 and has focused on for enforcement in 2019
- The nuances in compliance areas that create risk and should be closely monitored in your quality system
- The differences and revisions in inspectional programs affecting all medical device regulatory and quality professionals
- Enforcement tools the FDA will be using in 2019 to ensure compliance and evolving trends in how the FDA seeks enforcement
- The business implications of FDA enforcement trends and compliance policy through its new initiatives

1:30 p.m. – 3:00 p.m. EST

Jobs via Kelly Services

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<tr>
<td>Scientist I</td>
<td>Pleasanton, CA</td>
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<tr>
<td>Health Program Representative</td>
<td>Denver, CO</td>
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<tr>
<td>Laboratory Technician (Entry-Level, 3rd shift)</td>
<td>Waltham, MA</td>
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<tr>
<td>Clinical Study Assistant</td>
<td>Santa Ana, CA</td>
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<tr>
<td>Label Coordinator (regulatory)</td>
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<tr>
<td>Senior Manager, Trial Safety</td>
<td>Summit, NJ</td>
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<tr>
<td>Study Data Manager</td>
<td>South San Francisco, CA</td>
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<tr>
<td>Fellowship - HIV</td>
<td>Foster City, CA</td>
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<td>Manufacturing Associate</td>
<td>Union City, GA</td>
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<tr>
<td>Marketability / Benchmarking Engineer</td>
<td>Farmington Hills, MI</td>
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<tr>
<td>Clinical Technologist</td>
<td>Saint Paul, MN</td>
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<tr>
<td>Clinical Supply Planner</td>
<td>Lansdale, PA</td>
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<tr>
<td>Senior Manager/ AD of Biostatistics</td>
<td>South San Francisco, CA</td>
</tr>
<tr>
<td>Registered Nurse</td>
<td>Blanding, UT</td>
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More Jobs

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<tr>
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<tbody>
<tr>
<td>Clinical Forecasting Specialist</td>
<td>Bristol-Myers Squibb, Princeton, NJ</td>
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<tr>
<td>Clinical Research Coordinator III</td>
<td>Emory, Atlanta, GA</td>
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<tr>
<td>Senior Clinical Research Coordinator</td>
<td>Elligo Health Research, Everett, WA</td>
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<td>Clinical Trial Project Manager</td>
<td>Monitored Therapeutics, Inc., Dublin, OH</td>
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<td>Clinical Trial Specialist</td>
<td>PRA Health Sciences, Lenexa, KS</td>
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<td>Technology Support Manager</td>
<td>Crmed, Durham, NC</td>
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<td>Clinical Research Associate</td>
<td>DOCS, Los Angeles, CA</td>
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<tr>
<td>Regulatory Affairs Coordinator</td>
<td>Virginia Commonwealth University, Richmond, VA</td>
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