Using patient registries to track effectiveness of cell and gene therapy trials

Due to advances in rare-disease research and individualized cell and gene therapies, there has been a recent crop of treatments approved by regulatory agencies not based on the “gold standard” randomized, controlled, Phase 3 clinical trial. Rather, drug companies are getting products on the market from small, Phase 2 trials with a single arm—those that treat all patients with the experimental therapy rather than having some on a placebo or standard-of-care treatment.

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“As we are going to be using more therapies that don’t lend themselves to the traditional randomized, controlled trials, we need to have a mechanism that allows us to critically assess the response and the safety profiles of these therapies,” says Partow Kebriaei, a medical oncologist.

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Kebriaei and other clinicians are creating patient registries to track the long-term outcomes of their patients who are participating in these small clinical trials or are being treated with a therapy that was approved based on a single-arm study.

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There are issues to consider, says Kebriaei, including who should have access to such datasets, how to protect patient privacy, and how industry and academia can collaborate on registries. “Also, how do we follow patients longitudinally as they move from institution to institution? We need a mechanism by which do to that.”

Read full, original post: Patient Registries to Bolster Cell- and Gene-Therapy Clinical Trial Data