How Real World Evidence Can Ease Payers' Anxieties About Orphan Drugs

In recent years, health insurers have argued that the high price tags on drugs for rare diseases are unsustainable.[1] It's not just that the drugs are too expensive, payers say. There are so many such products in the pipeline that multiple approvals could result in a rapid depletion of available funds for reimbursement. That's why, at America's Health Insurance Plans (AHIP), an influential trade association, reducing tax deductions and other incentives to develop rare disease medicines under the 1983 Orphan Drug Act (ODA) is a top agenda item.[2] And, partly in response to alarms payers have sounded, the 2017 tax overhaul enacted by Congress includes a 50 percent cut in ODA tax deductions.[3]

To learn more about the scenarios that worry health insurers and the steps biopharmaceutical companies might take in response, Syneos Health interviewed executives at managed care organisations and integrated delivery networks representing 47.2 million covered lives. Among other issues, which Syneos Health summarised in a December report,[4] payers voiced their concerns that product manufacturers published inadequate data defining long-term outcomes, cost offsets (such as reductions in hospitalizations), post-marketing surveillance.

Many payers also contended that patient advocacy organisations working in rare diseases have been steered down unfruitful paths by their manufacturing partners. Logically, according to health insurers, patient advocates should lobby for lower drug prices. Instead, payers told us desire for access to novel therapies as well as financial dependence on manufacturers often compels patient organisations to support their industry partners in the quest for accelerated drug approval and favourable formulary placement – with or without a strong evidentiary case.

When Syneos Health shared summaries of payer concerns with advocacy groups working in rare diseases, the advocates objected to the payers' characterisations of the problem. In short, patient advocacy groups believe innovations in the drug pipeline and in the clinical trial process will apply downward pressure on rare disease prices over time, defusing the financial crisis payers believe to be imminent.

Advocacy responses to payer concerns about orphan drug prices will likely resonate with anyone who is actively involved in testing rare disease medicines. Their case is especially persuasive when it comes to innovations in real world evidence (RWE), analytics, and clinical trial design.

Post marketing studies and RWE generation are not new concepts, however there is a more recent and growing emphasis on these sorts of data, particularly in rare diseases. The trend is especially promising when RWE signals are amplified by more sophisticated tools that can be deployed to mine heterogeneous heath databases, and by the fast-expanding catalogue of personal health apps and devices connecting patients to medical professionals. With these advances, researchers can only feel inspired to redouble our efforts and use every tool at our disposal.

The characteristics of Rare Diseases mandate small trials enrolling small numbers of patients. Researchers in Rare Diseases must leverage RWE and advanced analytics to design more patient centered trials that can enroll the limited number of patients instead of the traditional model of designing a trial then looking for patients that are eligible for enrollment.

The good news for patients, for payers, and for all healthcare stakeholders, is that the US government supports innovation – both in developing new evidence modalities and novel clinical trial design. The 21st Century Cures Act encourages us to experiment with RWE in clinical decision support, and the US Food and Drug Administration (FDA)'s Critical Path initiative supports the use of interim data analysis to enable adjustment of sample size, modification of dosing protocols, and termination of inferior treatment arms.

Further innovations now beckon. Different computer systems housing insurance claims data and electronic medical records can be accessed to identify and recruit patients in clinical research, from Phase I straight through post-marketing surveillance. The FDA seems increasingly open to pragmatic clinical trials comparing two or more drug regimens in real-world settings to inform doctors' decision-making. Finally, FDA advisory groups, watching the swift evolution of biosensor-enhanced wearables and smartphone health apps, are starting to consider regulatory pathways that incorporate these sorts of real world, patient generated data.

With these developments and many more on the near- and mid-term horizon, the promise of RWE informing clinical research and supporting economic claims that.

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