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We need to understand what a potential cure really means, whether the benefits last one or two years or extend throughout a patient's lifetime

26.11.2026

Tags: USA, Kaiser Permanente, Rare Diseases, Insurance, Access

For rare disease patients, the promise of a new therapy raises questions that extend well beyond regulatory approval, from identifying those most likely to benefit to determining whether its effects will last. Dr Maisha Draves, associate executive director of The Permanente Medical Group, examines how Kaiser Permanente navigates these uncertainties through value-based, integrated care, clinical evidence and long-term planning, while confronting the financial and operational realities of cell and gene therapies.

How does Kaiser Permanente’s integrated care model support the early identification of patients with rare diseases?

One of the advantages of an integrated, value-based care system is that we have a clear picture of our patients throughout their lives, from maternal care and birth through newborn screening and ongoing care. At Kaiser Permanente, our electronic health records connect primary care physicians, specialists, subspecialists, geneticists, laboratory services, imaging and pharmacy, allowing us to identify symptoms or delays in developmental milestones earlier and connect patients with the appropriate care. Whether that involves a primary care physician, a specialist or further evaluation and genetic testing, having that continuity of information helps us recognise when additional expertise may be needed.

We also provide genetic screening and counselling during maternal care, alongside newborn screening in our hospitals and further diagnostic testing when indicated. This allows us to follow patients across different stages of care and respond as their needs evolve, rather than looking at each interaction in isolation. As clinical evidence develops and our understanding of rare diseases advances, we continue to refine how we identify patients and ensure they receive the right care at the right time.

What role do genomics and specialist expertise play in Kaiser Permanente's approach to rare disease care?

Genomics is an evolving field, and we continue to adapt as new tests and therapies become available. Our physicians and pharmacists work together to review the clinical evidence, identify the right patients and develop processes to match them with appropriate treatments. Across our Permanente Medical Groups, we draw on the expertise of specialists, subspecialists and geneticists throughout the system to develop clinical tools, educational resources and timely recommendations. This is especially important for rare conditions, where specialist expertise may be difficult to find, and helps physicians recognise when a patient may benefit from additional consultation or genetic evaluation.

We also work closely with our laboratory and pharmacy teams to review emerging evidence and incorporate relevant specialty society guidelines into our clinical workflows, ensuring that physicians have access to clear, evidence-based recommendations. By sharing this expertise more widely, we can support primary care physicians and specialists when they encounter conditions they may rarely see in practice. Where a patient's needs extend beyond the expertise or services available within our system, we can also work with external partners to arrange care at a quaternary centre when necessary.

How does Kaiser Permanente assess rare disease therapies when clinical evidence is limited, and what role does real-world data play?

We are excited about the innovation in rare disease treatments, particularly for conditions that, when I was in medical school, we learned about but had very little to offer patients. However, given the small patient populations and limited clinical trials, we need to carefully consider both what the evidence tells us and what remains uncertain about a treatment's efficacy and safety. Our

pharmacy subject matter experts work alongside Permanente physician specialists and subspecialists to evaluate the available data, examining who was studied, who is most likely to benefit and the magnitude and clinical significance of that benefit. We look at whether the results reflect changes in biomarkers or meaningful clinical improvements, such as delayed disease progression, while considering how well the findings apply across different forms and stages of a rare disease.

Many of these therapies rely on biomarker outcomes and relatively short follow-up periods, leaving important questions about the durability of their benefits, long-term safety and potential complications that may emerge later. This makes real-world evidence and continued monitoring essential to understanding how treatments perform beyond clinical trials and throughout patients' lives. It is a shared responsibility across the healthcare ecosystem, with manufacturers continuing to generate long-term evidence, the FDA overseeing postmarketing monitoring, and healthcare systems contributing their clinical findings and outcomes. Patients and caregivers are also part of this ongoing process, as we continue to learn about the benefits, risks and uncertainties associated with these therapies.

What makes a productive discussion with manufacturers when evaluating high-cost rare disease therapies?

For any rare disease therapy, discussions with manufacturers need to go beyond the price of the drug itself. As an integrated healthcare system, we need to understand the clinical evidence, the patient population and the different stages of disease progression to determine where a treatment may fit best, while recognising the uncertainties that remain around its long-term efficacy and safety. We also need to consider everything involved in delivering that treatment, from diagnostics and genetic testing to the initial costs of administration, ongoing monitoring and long-term follow-up. A patient may continue to require care for their underlying condition, even after receiving treatment, as well as for any side effects or safety concerns that emerge over time. Understanding these broader healthcare needs, alongside the durability of treatment outcomes, is essential to ensuring that discussions around pricing reflect the full cost of caring for a patient throughout their life, rather than the cost of a single therapy.

How does Kaiser Permanente anticipate and prepare for emerging advanced therapies ahead of regulatory approval?

Our planning process begins well before approval by the FDA, with our physicians and pharmacists continuously monitoring the development pipeline, including therapies in early-stage clinical trials. We typically look about five years ahead to understand the potential clinical, operational and financial implications of emerging treatments, because waiting until approval could mean missing the opportunity to identify the right patients and provide the right care at the right time. As new evidence emerges and therapies progress through regulatory review, our teams independently evaluate the available data to determine which patient populations are most likely to benefit, what uncertainties remain around efficacy and safety, and what monitoring may be required. We also consider potential interest in broader or off-label use, working closely with physicians, pharmacy teams and patients to ensure that treatment decisions remain grounded in the evidence.

Beyond the clinical considerations, we look at the operational complexities of introducing these therapies, whether that involves specialised storage, different supply chains or new delivery systems, while also assessing competing treatments already available or in development. Understanding these requirements early allows us to anticipate the resources and preparations needed before a therapy reaches the market. Our goal is to be proactive in bringing together the clinical evidence and operational planning, ensuring that our physicians have the tools and recommendations to follow best practices and make informed decisions with patients, particularly when important questions about a treatment’s benefits and risks remain unanswered.

How is Kaiser Permanente approaching the financial challenges of one-time cell and gene therapies, and what payment models could support their long-term sustainability?

This is where being an integrated, value-based care system, with a large membership base and a broad network of physicians and pharmacy teams, gives us an advantage, as we can work together from the outset to plan for the clinical, operational and financial implications of one-time cell and gene therapies. Budgeting for these treatments begins with understanding the evidence, identifying the right patients for the right indications and ensuring that we have the necessary expertise, centres of excellence, infrastructure and contracting arrangements in place. We also need to understand what a potential cure really means, whether the benefits last one or two years or extend throughout a patient’s lifetime. That question of durability is critical, because the price of the therapy is only one part of the picture, and the entire system needs to work together to ensure that patients receive the greatest possible benefit.

There is also a broader national discussion about how to make these therapies financially sustainable, including risk pooling and outcomes-based instalment payments that link reimbursement to clinical results over time. These models could account for the success of the initial treatment, clinical outcomes after one or two years and the durability of those benefits over three to five years. I think it is encouraging that governments and healthcare systems are exploring these approaches, particularly as the financial challenges of rare diseases can be difficult for any single system to manage alone. The question is how we can develop models that ensure patients receive the care and coverage they need while allowing healthcare systems across the country to sustain the delivery of these treatments over the long term.

Where do the greatest challenges lie in delivering advanced therapies, and how is Kaiser Permanente addressing them?

These advanced therapies require healthcare systems to prepare for much more than the treatment itself. We need to ensure that our primary care physicians, specialists, subspecialists, geneticists, pharmacy and laboratory teams, hospitals and health plan are all working together, with the right clinical expertise and evidence-based processes in place. This involves coordinating every stage of care, from screening, testing and diagnosis to the clinical, operational and financial infrastructure needed to deliver treatment, while ensuring that patients, their families and caregivers receive the support they need throughout the process and during long-term follow-up.

This is a highly specialised area, and we continue to develop the expertise and infrastructure required, including appropriate treatment centres, specialised handling and procurement processes, and coordination with manufacturers. At the same time, the evidence is still evolving, with important uncertainties around long-term efficacy, durability and potential side effects, which makes continued research and monitoring essential. Ultimately, we need to ensure that the entire clinical team is working together to coordinate each treatment and support patients well beyond its initial delivery.

What should healthcare systems prioritise as access to innovative rare disease therapies continues to expand?

We are excited about the growing availability of rare disease therapies, but we also care deeply about patient safety, meaningful clinical evidence and ensuring that the right patients receive the right treatments. The recent FDA investigation of onasemnogene abeparvovec (Zolgensma), a

gene therapy for spinal muscular atrophy (SMA), illustrates this challenge. While the therapy has brought significant benefits to patients, the risk of tumour development associated with adeno-associated viral (AAV) vector integration, previously considered theoretical, has now emerged as a real-world safety concern, with the FDA confirming product-related insertional oncogenesis in two cases. This reinforces why we need to continue building evidence and monitoring patients over the long term, particularly when therapies are initially approved with limited data. Everyone across the healthcare system needs to take the durability of both clinical benefits and safety seriously, maintaining the same vigilance in postmarketing surveillance as we do when these treatments first become available.

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