

Domain 2 Findings - Access to Current Treatments

- Respondents listed quantitative and qualitative data and perspectives that would be most helpful to inform state Medicaid directors to ensure that utilization management approaches are not across-the-board policies that inadvertently result in care delays for individuals with SCD.

Quantitative data examples that were identified are detailed below.

- **Costs and outcomes data.** Multiple respondents suggested data explaining how the costs and outcomes related to SCD would justify some exception to normal protocols (e.g., how outcomes and costs are impacted if individuals with SCD do or do not get needed care).
- **Effect of barriers to care.** Multiple respondents suggested data explaining how individuals with SCD face specific barriers to care (including medical and care coordination, access to reliable transportation, etc.).
- **Prevalence, care utilization.** Respondents noted the importance of basic information regarding statistics on prevalence, information on the settings of care SCD patients utilize, and the types of clinicians they see.
- **Prior authorization.** Multiple respondents suggested data on prior authorizations would be helpful, including which specific prior authorizations tend to interfere with care, comparative data on patient experiences/cost/outcomes for individuals with treatments under prior authorization, and data on the many times prior authorizations are denied or approved (because a treatment consistently approved can have the prior authorization removed). It was also suggested to conduct a comparative analysis of relative savings from utilization management and the negative impact to patients (such as potential additional costs due to poor care management).
- **Other respondents suggested a range of data that could be impactful,** including: data on delays in receiving medication or treatment, and the effect the delays have on overall health and care; data on how treatment can improve length of life; estimates of intervention costs; data regarding which evidence-based practices can inform utilization management policies.

Qualitative examples that were identified are detailed below.

- **Multiple respondents highlighted the importance of speaking with patients and parents of patients,** including through fact-based, lived experience testimony to help program leaders know what living with SCD is like.

- **Medicaid managed care plans.** It was noted that Medicaid agencies should create specific access specifications in the MCO contracts. Such requirements could be measured and tracked publicly, which would create incentives for positive access and outcomes. One concern is the potential lack of specificity in requests related to prior authorization denials and appeals, saying that managed care plans would need to be given/request more information.
- **Other respondents suggested a range of approaches including:** a presentation from a neutral SCD expert (e.g., researcher unaffiliated with drug manufacturers) about the impacts of SCD on individuals and the benefits of appropriate treatment; articulating an approach to reforming utilization management policy that is generalizable and allows for appropriate variability to address negative impacts on individuals with rare diseases including, but not limited to, SCD; specific actions on how P&T committees can help; explaining why the risk of individuals with SCD seeking inappropriate care are low; work with Medicaid managed care plans and SCD specialty providers to minimize prior authorization requirements for patients of certain vetted providers.
- **Respondents listed quantitative and qualitative data and perspectives that are most helpful to improve a Medicaid Pharmacy and Therapeutics (P&T) committee’s understanding of, and responsiveness to, SCD in a manner that results in improved access to care and treatment for individuals with SCD.**

Quantitative data examples that were identified are detailed below.

- **Published research and literature.** Multiple respondents noted the importance of peer-reviewed studies on the efficacy of different treatments, noting the importance of “rely[ing] heavily on published literature.” Respondents noted the importance of evidence-based best practices and outcome data, as well as cost effectiveness data. They also emphasized the importance of highlighting any clinical data and guidelines for treatment that demonstrate that novel SCD therapies are unlikely to be misused.
- **P&T Committee metrics.** Stakeholders proposed metrics that P&T committees can track as a standing agenda item so the Committee can identify barriers and assess improvement over time.
- **Impacts on individuals’ lives.** Respondents noted the importance of data highlighting quality of life, length of life, and the ability to go to school and hold a job as a contributing member of society.

Qualitative data examples that were identified by respondents are detailed below.

- **Early engagement with clear education, evidence.** Respondents emphasized that stakeholders should help to educate the agency or support staff charged with the drafting of measures. They noted that it is often too late for advocacy efforts once a measure is up for a vote at a committee meeting. Stakeholders should also consider educational sessions with state

Medicaid staff (clinical and financial leadership, and P&T committees), in which a neutral SCD expert (e.g., a researcher unaffiliated with drug manufacturers) gives a presentation about the impacts of SCD on individuals and the benefits of appropriate treatment. These stakeholders can also help to facilitate access to credible, distilled, and rigorous summaries of the timely evidence that should inform P&R deliberations around SCD policy.

- **Tailored care management.** Respondents explained that information can be provided to Medicaid directors to help establish tailored requirement of care plans and/or establish practices for chronic disease management. This could involve requiring certain staff to be trained in successful SCD pain management and/or requiring the P&T committee to review utilization data and prior authorization policies.
- **Personalized, localized.** Bring together stories from individuals in the state about living with SCD and the difficulties in accessing appropriate treatment.
- **Respondents assessed the extent to which it is important that state Medicaid programs adopt approaches to utilization management and P&T committees that take into account the clinical needs and personal preferences of individuals living with rare diseases such as SCD.**
 - Most respondents assessed this goal as “very important” or “important.”
- **Respondents relayed their sense of the extent to which patient advocate testimony in P&T Committee meetings is considered when making subsequent P&T decisions.**
 - Most respondents who had experiences with patient advocate testimony said such perspectives are “strongly considered,” “considered,” or “somewhat considered.”
 - Several respondents had not had experiences with patient advocate testimony in P&T meetings, so they felt they could not comment.
 - Two respondents said such perspectives were not considered.

