United States Senate
COMMITTEE ON HEALTH, EDUCATION, LABOR, AND PENSIONS
WASHINGTON, DC 20510–6300

December 5, 2023

To Interested Parties:

Cell and gene therapies have incredible curative potential for our most vulnerable patients, but their access is limited by a health care market that was not designed to pay for them.

Given the current limited number of approved gene therapies, manufacturers and commercial health plans alike are currently able to absorb costs in order to provide patient access. But gene therapy approvals are expected to grow dramatically over the next decade. While this will give American patients more cures to deadly diseases, it will also pose a serious financial risk to health plans, which could dissuade them from covering these groundbreaking curative treatments.

For patients with an ultra-rare disease, the stakes are even higher: artificially low prices could promote patient access, but eliminate the incentive to bring these treatments to market. Conversely, the small patient population runs the risk of bearing the full weight of the high price tag. While progress has been made in promoting access to access to cell and gene therapies more broadly, the small size of the ultra-rare disease population makes access solutions particularly challenging and supports a greater role for Congress in shaping those solutions. Further, any proposal put forward by Congress should be informed by the current work of states and the private market.

If a patient cannot afford innovative treatments, to them it is as if that innovation never occurred. Policymakers have a responsibility to evaluate the market structure and identify where targeted changes can be made to ensure market access for patients with ultra-rare diseases. As we examine steps to ensure access of these therapies for patients with an ultra-rare disease, we request feedback on the topics and questions below. Please submit any responses to GeneTherapyCoverage@help.senate.gov by **January 22, 2024.**

Which Treatments Should Be Included?

1. How should lawmakers define an “ultra-rare” disease or disorder cell or gene therapies should be eligible for inclusion in new coverage or contracting requirements for those patients with an ultra-rare disease or disorder? What definitions should lawmakers consider?

2. Are there other criteria that lawmakers should consider in determining which therapies should be included in new coverage or contracting models? Examples could include treatment characteristics (e.g. curative treatments or treatments reaching a certain cost threshold) or treatments fitting certain patient profiles (e.g. pediatric patient populations or the fatality of the disease) If so, what definitions should lawmakers consider?

What is the Current Practice for Patients with Ultra-Rare Diseases or Disorders?

3. How do patient populations currently access and pay for these therapies?

4. What, if any, federal or state programs do these patient populations use as they seek to pay for and access these therapies and related care? What is the specific benefit to the patient in using these programs? For example, interested parties could contemplate instances where families choose to "spend down" to become eligible for state Medicaid programs, thus ensuring coverage.

5. What, if any, manufacturer-sponsored programs do these patient populations currently use to access and pay for these therapies? How do patient populations apply for or access these programs?

6. What, if any, other privately-sponsored programs do these patient populations currently use to access and pay for these therapies? How do patient populations apply for or access these programs?

7. What, if any, are the utilization management tools (e.g. step therapy, prior authorization) that patients are typically subject to when paying for and accessing these therapies? If not the patient, what individual or entity typically works through the process of obtaining approvals?

8. What, if any, are the cost-sharing mechanisms that patients are typically subject to when paying for and accessing these therapies?

9. How is access and pricing of these therapies communicated between patient, physician, manufacturer, and payer? What, if any, barriers or challenges exist as parties seek to communicate access and pricing with one another (e.g. timeliness, accurate accounting of costs, understanding of benefits, financial risk)?

10. How do vulnerable patient populations demonstrate financial need as they seek to pay for and access these therapies? If not listed as part of the above, what other mechanisms exist to support access for patients who have demonstrated financial need?

How Do Plans and Payers Currently Manage Financial Risk?

11. What does coverage for these therapies typically look like? What does the landscape look like for coverage of these therapies?

12. What are the typical elements of a benefit design that includes coverage of these therapies?

13. What factors do benefit consultants consider when designing benefits that include coverage of these therapies?

14. What factors do health plan brokers consider when selling health insurance products to individuals or entities looking to include coverage of these therapies?

15. What contract options exist for health plans and other payers to mitigate the cost of covering these therapies?

16. What mechanisms or tools do health plans and other payers currently use to manage the cost of covering these therapies?

17. What factors or challenges do health plans and other payers typically consider when determining whether to cover one or all of these therapies?

18. What are the typical characteristics of health plans and payers who choose to not cover these therapies? What about for those who do cover these therapies?

19. How do health plans and other payers currently evaluate the long-term financial benefit of covering these therapies compared to the high up-front cost? Interested parties should include information on how they evaluate these therapies in relation to existing clinical treatments.

20. How do health plans and other payers currently evaluate the financial benefit of covering these therapies in instances where a patient might switch health plans?

21. If separate from financing the cost of the therapy, such as for associated wrap-around services, please describe how health plans and other payers manage the costs of administering the therapy and associated care.

22. What role does third-party assistance or manufacturer assistance play in financing these therapies?

23. Please share any other relevant information in regard to health plan or other payer coverage of these therapies.

How do Manufacturers Price and Design Contracts for Treatment of Ultra-Rare Disease?

24. Please share non-proprietary pricing strategies and mechanisms drug manufacturers use to set list prices for these therapies.

25. Are there typical contract designs between the drug manufacturer and the health plan or payer (or, if applicable, the intermediary negotiating contracts on behalf of the health plan or payer) for these therapies?

26. What role, if any, do manufacturer rebates play in negotiating contracts between the drug manufacturer and the health plan or payer for these therapies?

27. What, if any, alternative contract designs do health plans or payers use to support coverage of these therapies?

28. Please share any other relevant information in regard to manufacturer market access strategy of these therapies.

How Do Supply Chain Intermediaries Price and Design Contracts?

29. What are typical contract designs between the drug manufacturer, wholesaler, and distributor as they seek to provide access to these therapies?

    What special supply chain considerations have to be made for these therapies as the drug manufacturer, wholesaler, and distributor seek to distribute these therapies?
30. How are administrative fees set within the contracts between drug manufacturer, wholesaler, and distributor as they seek to provide access to these therapies?

31. What percent do these administrative fees make up as part of the total cost of these therapies?

32. What are typical contract designs between wholesaler, distributor, group purchasing organization (GPO), health provider, and pharmacy as they seek to provide access to these therapies? For instance, interested parties could contemplate the value provided by exclusive or nonexclusive contracts between two entities.

33. Please share any other relevant information in regard to the role supply chain intermediaries play in providing access to these therapies.

How Do Physicians Provide Access to These Therapies?

34. How does a physician or health system initiate the process of prescribing a patient with an ultra-rare disease or disorder one of these therapies?

35. Do physicians or health systems bear any financial risk as part of prescribing a patient with an ultra-rare disease or disorder these therapies? If so, as part of what program or what type of contract?

36. What is the typical communication between the physician, health system, and manufacturer as a part of prescribing a patient with an ultra-rare disease or disorder these therapies?

37. What is the typical communication between the physician, health system, and health plan or payer as part of prescribing a patient with an ultra-rare disease or disorder these therapies?

38. Do physicians or patients with an ultra-rare disease or disorder use a dispensing channel similar to other physician-administered treatments to access these therapies, or is there an alternative method?

What is the Future of Access for These Therapies?

39. What is the appropriate role of the federal government in ensuring access to these therapies in the commercial market? How can any steps taken on the federal level ensure expanded access while not hurting innovation in this area?

40. Should the federal government mandate coverage of these therapies? What markets (e.g. small, large group markets) or plans should be required to cover these therapies?

41. What are the anticipated costs or savings to health systems, plans, payers, or patients as a greater number of these therapies become available?

42. How should anticipated benefits from these therapies be evaluated against the potential costs of these therapies?

43. How should these therapies be financed?

44. How can future payment or coverage models for these therapies be designed in a way that drives down total health costs for the patient?

45. Which entity should accept the majority of the financial risk when providing access to these therapies? Why?

46. What role should utilization management tools play in providing access to these therapies?

47. How quickly should these covered therapies be made available to patients?

48. What other considerations should be made around benefit design to ensure access to these therapies (e.g. deductibles, cost-sharing)?

49. Should health care providers share in the financial risk of prescribing these therapies to patients? Why or why not?

50. What role should patient assistance programs play in providing access to these therapies?

51. Are additional regulatory requirements or flexibilities needed to promote health plan or payer coverage of these therapies?

52. How should policymakers consider other eligibility criteria for access to these therapies for populations such as individuals with long-term disabilities or complex medical needs who are eligible for Medicaid based on disability? What role should commercial insurance play in the long-term for covering these patients who may no longer have the disability that made them Medicaid eligible?

53. Please provide feedback on payment and contracting options for health plans, payers, and manufacturers that would provide access to these therapies for patients. These contract options could include value-based models, warranties, annuities, shared savings models, or other risk-based contracting models. Please provide any relevant examples based on existing models.

<u>How Should Federal or State Governments Promote Access to New Models?</u>

54. How could federal and state governments leverage existing waiver authorities in order to promote commercial access to these therapies? For instance, interested parties could contemplate changes to the existing waivers in Section 1332 of the Patient Protection and Affordable Care Act (ACA) which could allow states to provide new payment models for these therapies in the small group market and in the individual market.

55. How could the federal government leverage existing alternative coverage models in order to promote commercial access to these therapies? For instance, interested parties could contemplate changes to independent, noncoordinated excepted benefits, which could allow health plans and payers to subsidize add-on benefits for these therapies.

56. How could the federal government modernize existing health insurance requirements in order to promote access to these therapies? For instance, interested parties could contemplate modifications to the portability requirements under the Health Insurance Portability and Accountability Act (HIPAA) which could allow patients to take their policy from plan to plan. In addition, interested parties could contemplate modifications to Essential Health Benefit (EHB) requirements to ensure coverage of these therapies.

57. How could the federal government promote greater transparency and competition amongst intermediaries to promote access to these therapies? For instance, interested parties could contemplate how greater flexibility in contracting could impact coverage of these therapies. In addition, interested parties could contemplate how different statutorily-defined pricing mechanisms (e.g. Average Wholesale Price, Average Sales Prices, Wholesale Acquisition Cost) might need to be adapted in order to promote access to these therapies.

<u>How Should Lawmakers Seek to Evaluate and Accomplish these Policy Goals?</u>

58. What are the tradeoffs of expanding coverage of certain therapies in one market over another? For instance, interested parties could contemplate the strategic benefit of prioritizing a new coverage model for a certain market (e.g. small group or large group) prior to deploying it more broadly.

59. What variables should lawmakers consider when evaluating which party should bear the greatest financial risk under different contracting or coverage models?

Sincerely,

Bill Cassidy, M.D.

Bill Cassidy, M.D.
Ranking Member
U.S. Senate Committee on Health,
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