Supplemental Material

Targeted Literature Review of Cost-Effectiveness Analyses in Sickle Cell Disease: Identifying the Optimal Modeling Approach for Therapies Targeting Sickle Hemoglobin Polymerization

Alexandra Taylor[1], Matthew Stargardter[2], Christine L Baker[3], Giovanna Tedesco Barcelos[4], Sandra Milev[1]

[1]Evidera, San Francisco, CA, USA; [2]Evidera, Bethesda, MD, USA; [3]Pfizer Inc, New York, NY, USA; [4]Pfizer AG, Zurich, Switzerland

- The 5 distinct structured searches performed as part of the targeted literature review included:
  - Google Scholar: ("cost effectiveness model" OR "cost utility model") (treatment OR therapy) "sickle cell disease";
  - Google Scholar: Allintitle: "cost effectiveness" OR "cost utility" OR CEA OR CEM OR CUA "sickle cell disease";
  - Google Scholar: Allintitle: "sickle cell" OR "Vaso-occlusive" OR "hemolysis" "cost effectiveness" OR "cost utility" -screening- "genetic testing";
  - PubMed: ((sickle cell disease [Title]) OR (SCD [Title])) AND (model) AND ((cost effectiveness) OR (cost utility)) NOT (screening [Title]);
  - Reference lists associated with existing literature reviews: Voxelotor NICE submission,[1] Purser et al 2020,[2] and Jiao et al 2021.[3]

Supplemental Figure: Sample Markov IPS model of SCD

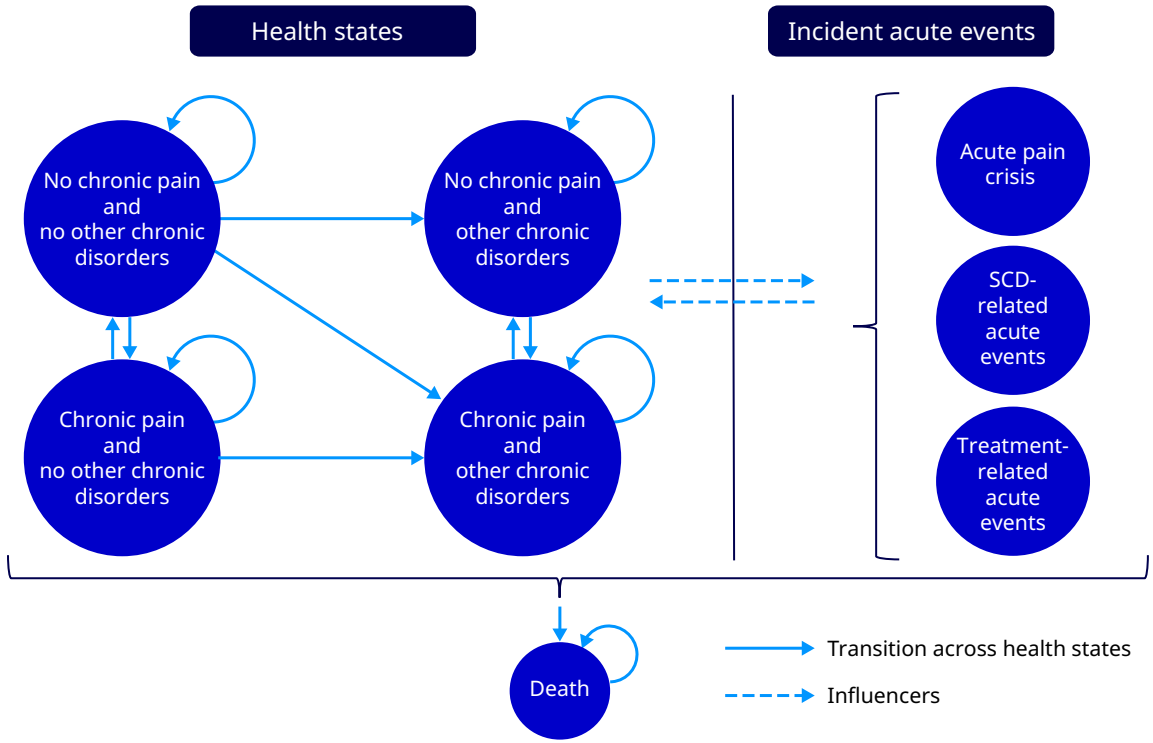

Diagram of the conceptual model of SCD. Treatment-related acute events include leukopenia, thrombocytopenia, and oligospermia/azoospermia associated with hydroxyurea; iron overload, transfusion reaction, and infection associated with transfusion; graft vs host disease, graft failure, posttransplant lymphoproliferative disorder, and secondary malignancy associated with hematopoietic stem cell transplant; and graft failure, posttransplant lymphoproliferative disorder, and secondary malignancy associated with genetic therapies.[4]

Abbreviations: CEA=cost-effectiveness analysis; CEM=cost-effectiveness model; CUA=cost-utility analysis; IPS=individual patient simulation; NICE=National Institute for Health and Care Excellence; SCD=sickle cell disease

References: 1. National Institute for Health and Care Excellence. Single technology appraisal. Voxelotor for treating haemolytic anaemia caused by sickle cell disease [ID1403]. Accessed March 20, 2024. https://www.nice.org.uk/guidance/gid-ta10505/documents/committee-papers-2. **2.** Purser M, et al. Value Health 2020; 23(S2). doi: 10.1016/j.jval.2020.08.098. **3.** Jiao B, et al. Pharmacoeconomics 2021;39:1225–41. **4.** Johnson KM, et al. PLoS One 2022;17:e0267448.