



## Advisory Council on Rare Diseases and Personalized Medicine

<u>Date</u>	<u>Time</u>	<u>Location</u>
October 11, 2023	10:30am – 2:00pm (CST)	Howerton Building, Room 202 615 Howerton Court Jefferson City, MO 65109 OR WebEx

[\\* Click HERE for Meeting Documents \\*](#)

### **Board Members Present**

Christopher Oermann, MD  
Claire Elson, PharmD  
Francis Cole, MD  
Gerald Wyckoff, PhD  
Jennifer Heeley, MD  
Jonathan Cooper, PhD  
Jonathan Wagner, DO  
Matthew McLaughlin, MD, MS  
Rosemary Britts  
Sherry Graf, RN, BSN  
Shivarajan Amudhavalli, M.B.B.S

### **Board Members Absent**

Michael Burke

### **MO HealthNet Staff Present**

Ambra Stotter-Bauda, Pharmacy Specialist  
Carmen Burton, Pharmacy Support Assistant  
Danielle Dodd, Special Counsel  
Elizabeth Short, Program Development Specialist  
Elizabeth Sissom, RN, Clinical Review Nurse  
Jennifer Colozza, PharmD, Clinical Pharmacist  
Joshua Moore, PharmD, Director of Pharmacy  
Chelsea Hammond, Pharmacy Benefit Specialist  
Lisa Smith, Pharmacy RFP Coordinator  
Mark Roaseau, R.Ph., Clinical Pharmacist  
Nikki Ashley, Pharmacy Program Specialist  
Olivia Rush, PharmD, Program Integrity Pharmacist  
Timothy Kling, MD, Acting Medical Director

### **Contractors Present**

Amanda Williams, PharmD, RPh, GWT  
Ashley Lytton, RN, BSN, Wipro  
Brandon Schowengerdt, PharmD, Conduent  
Chelsea Pendleton, RN, BSN, Wipro  
Karen Powell, PharmD, MS, GWT  
LeAnna Kirsch, RN, Wipro  
Lynn Morsches, PharmD, Conduent  
Mandy Nilges, RN, Wipro  
Megan Fast, PharmD, GWT  
Sandy Kapur, PharmD, GWT  
Serena Barden, PharmD, BCPS, GWT

**Others Present:**

Amy Galligher

Andi Stratton

Brian Berg

Cami Bizal

Danny Galligher

Dominic Marchese

Gary Parentuea

Gene McCarty

Jason Enders

Jodi Jensen

Joe Payne

Johnathan Leesman

Jon Joseph Vlasnik

Kapil Arya

Kate Ogden

Kelly Guntrum

Kim Eggert

Mark Stephens

Robert Jaramillo

Scott

Susie Moroney

DRAFT

<b>Welcome, Announcements and Introductions</b>	Josh Moore, MHD Pharmacy Director, introduced himself, and Brandon Schowengerdt, Conduent's new Clinical Pharmacist. Claire Elson, Council Chair, introduced Cami Bizal, Children's Mercy PGY1 Pharmacy Resident. Josh called the meeting to order and facilitated the meeting on behalf of MHD.
<b>Minutes Review</b>	<p><b>Discussion:</b> Minutes were reviewed from the July meeting.</p> <p><b>Decision:</b> The Council voted to accept these approved minutes with no revisions.</p>
<b>Pharmacy Program and Budget Update</b>	<p>Elizabeth Short and Josh Moore presented information regarding the Pharmacy Program and Budget Updates.</p> <p>Information presented included:</p> <ul style="list-style-type: none"> <li>- July – August 2023 Enrollees and Expenditures</li> <li>- July – August 2023 MO HealthNet Expenditures by Service</li> <li>- July – August 2023 Pharmacy Spend vs Total Medicaid Spend</li> <li>- July – September 2023 Pharmacy Expenditures</li> <li>- July – September 2023 Pharmacy Specialty and Non-Specialty Expenditures</li> <li>- July – August 2023 Adult Expansion Group Expenditures and Participant Count</li> <li>- FY20 to FY24TD Mavyret Expenditures</li> <li>- FY24TD Top 10 Drug Classes by Expenditures</li> <li>- FY20 – FY24TD Rare Disease Expenditures Per Day</li> </ul>
<b>Old Business</b>	<p>Handouts for the Edit Implementation Schedule and criteria for Previously Approved Clinical Edits, Step Therapies, and Prior Authorizations will be posted to the Division's web page:</p> <p><a href="https://dss.mo.gov/mhd/cs/advisory/rdac/meeting.htm">https://dss.mo.gov/mhd/cs/advisory/rdac/meeting.htm</a></p>

**Sickle Cell Disease (SCD) Clinical  
Edit**

**Discussion:**

- Jennifer Colozza presented the “Sickle Cell Disease Annual Presentation”.
  - o Council asked about MHD’s thoughts on why some participants were not getting therapy.
    - It was explained that third party liability (TPL) participants could sway the results of the reporting as it could be assumed most other third parties fully covered SCD medications, therefore MHD would never see those claims.
    - It was also explained that Dr. King, with WashU, is developing a state action plan with input from MHD. All hope to see better access to care with the plans implementation.
  - o Council questioned the expected pricing of the forthcoming gene therapies.
    - It was explained that the price expectations are to be in the million to millions of dollars range. Discussion arose on the potential or non-potential of competition to help drive costs down.
- Jennifer Colozza introduced the proposal to the Council for discussion.
  - o Council asked if future proposals could include unique participant counts – MHD agreed this could be done for the Rare Disease or high-cost medication proposals.
  - o Council and MHD discussed the step-therapy requirements and those participants who are reluctant to take hydroxyurea due to potential side effects.
    - After discussion, all agreed to support the MHD DPAC and DUR board’s decision to utilize step therapy unless contraindicated or intolerance occurred.
- Susie Moroney, PharmD, with Novartis yielded time back.

**Decision:** The Council voted to accept the recommended criteria with no modifications.

**Spinal Muscular Atrophy (SMA)  
Clinical Edit**

**Discussion:**

- Josh Moore introduced the proposal to the Council for discussion.
  - o Council asked about the number of participants that have requested additional therapy after treatment with Zolgensma.
    - No specific numbers were available at the time. However, other Council members did discuss WashU utilizing dual therapy (i.e. either Spinraza or Evrysdi following Zolgensma treatment) while Children's Mercy only had one participant with dual therapy through a compassionate use program.
  - o Council and MHD discussed current and expected study results of ongoing clinical trials regarding Spinraza or Evrysdi therapy following Zolgensma treatment.
    - All agreed to continue to watch study results, case reports and other clinical information and discuss more at the next annual review. Council did provide some links to clinical studies.
    - MHD to stay the course in reviewing dual therapy requests.
  - o Council made suggestions to modify the wording found in the introductory paragraph.
- Danny Galligher, a parent of a SMA participant, along with the participant's physician, Kapil Arya, MD with Arkansas Children's Hospital, provided public comment.
- Jodi Jensen, PharmD, with Biogen provided public comment on Spinraza.
  - o Council asked if the manufacturer was still offering a compassionate use program.
    - It was explained that a compassionate use program was available, however, no additional details were available at the time.
- It was moved and seconded that the introductory paragraph be changed as follows:
  - o Unlike Spinraza and Evrysdi, which provide maintenance therapy aimed at slowing the progression of the disease, current evidence and guidance position Zolgensma as a one-time, single-dose IV infusion treatment intended to ~~repair the dysfunctional~~ **provide a functional copy of the** SMN1 gene.

**Decision:** The Council voted to accept the recommended criteria with the added introductory paragraph modifications outlined above.

<p><b>Neuromyelitis Optica Spectrum Disorder (NMOSD) Clinical Criteria</b></p>	<p><b>Discussion:</b></p> <ul style="list-style-type: none"> <li>- Josh Moore introduced the proposal to the Council for discussion. <ul style="list-style-type: none"> <li>o Council and MHD discussed the number of requests MHD has received for the respective medications and their individual costs.</li> <li>o Council and MHD discussed the possibility of utilizing multiple medications for a disease state when the medications have different mechanisms of action.</li> </ul> </li> <li>- Jason Enders, PharmD, with Horizon Therapeutics provided public comment on Uplizna.</li> </ul> <p><b>Decision:</b> The Council voted to accept the recommended criteria with no modifications.</p>
<p><b>New Business</b></p>	
<p><b>Generalized Myasthenia Gravis (gMG) Clinical Edit (formerly Vyvgart CE)</b></p>	<p><b>Discussion:</b></p> <ul style="list-style-type: none"> <li>- Jennifer Colozza introduced the proposal to the Council for discussion. <ul style="list-style-type: none"> <li>o Council briefly discussed the recommended criteria.</li> </ul> </li> <li>- Brian Berg with UCB, Inc. provided public comment on Rystiggo.</li> </ul> <p><b>Decision:</b> The Council voted to accept the recommended criteria with no modifications.</p>
<p><b>Skyclarys Clinical Edit</b></p>	<p><b>Discussion:</b></p> <ul style="list-style-type: none"> <li>- Josh Moore introduced the medication and proposal to the Council for discussion. <ul style="list-style-type: none"> <li>o Council and MHD discussed the modified Friedreich's Ataxia Rating Scale (mFARS) and the relevance to clinical practice. <ul style="list-style-type: none"> <li>▪ It was explained that all requests sent to MHD so far for Skyclarys included an mFARS when requested by the clinical reviewer.</li> </ul> </li> <li>o Council and MHD briefly discussed how the clinical trial length influenced the initial approval period recommendations.</li> </ul> </li> <li>- Robert Jaramillo with Reata Pharmaceuticals provided public comment on Skyclarys.</li> </ul> <p><b>Decision:</b> The Council voted to accept the recommended criteria with no modifications.</p>

<b>Vyjuvek Clinical Edit</b>	<p><b>Discussion:</b></p> <ul style="list-style-type: none"> <li>- Jennifer Colozza introduced the medication and proposal to the Council for discussion. <ul style="list-style-type: none"> <li>o MHD provided clarification on how the cost per month and year could change depending on real-world utilization of the medication.</li> </ul> </li> <li>- Dominic Marchese with Krystal Biotech provided public comment on Vyjuvek.</li> </ul> <p><b>Decision:</b> The Council voted to accept the recommended criteria with no modifications.</p>
<b>Motion to Close</b>	Matthew McLaughlin motioned for the meeting to be closed (see attached roll call).
<b>Other Business/ Rare Disease Topic of Discussion</b>	None at this time.

**NEXT MEETING: Wednesday, January 10, 2024;** 615 Howerton Court, Conference Room 202 Jefferson City, MO 65109 or WebEx

### Roll Call for October 11, 2023

Council Member	July 2023 Minutes	SCD Clinical Edit	SMA Clinical Edit	NMOSD Clinical Edit	gMG Clinical Edit	Skyclarys Clinical Edit	Vyjuvek Clinical Edit	Closing	Adjournment
Christopher Oermann	Y	MY	Y	Y	Y	Y	Y	Y	MY
Claire Elson	Y	Y	Y	MY	MY	SY	Y	Y	Y
Francis Cole	A	SY	Y	SY	Y	MY	MY	Y	Y
Gerald Wyckoff	A	A	A	Y	SY	Y	Y	Y	Y
Jennifer Heeley	Y	Y	Y	Y	Y	Y	Y	Y	Y
Jonathan Cooper	Y	Y	Y	Y	Y	Y	Y	Y	Y
Jonathan Wagner	Y	Y	Y	Y	Y	Y	Y	Y	Y
Matthew McLaughlin	Y	Y	SY	Y	Y	Y	Y	MY	SY
Michael Burke	A	A	A	A	A	A	A	A	A
Rosemary Britts	SY	Y	MY	Y	Y	Y	SY	Y	Y
Sherry Graf	MY	Y	Y	Y	Y	Y	Y	SY	Y
Shivarajan Amudhavalli	Y	Y	Y	Y	Y	Y	Y	Y	Y

Roll Call Abbreviations: A-Absent; AL-Alternate; R-Ratify; M-Motion; S-Second; Y-Yes; N-No; AB-Abstain