Advisory Council on Rare Diseases and Personalized Medicine

Date	Time	Location
February 8, 2022	10:30am – 2:00pm (CST)	Howerton Building, Room 202 615 Howerton Court Jefferson City, MO 65109 OR WebEx

*** Click HERE for Meeting Documents ***

Council Members Present:

Patricia Dickson, MD, Chair
Gerald Wyckoff, PhD
Claire Elson, PharmD
Christopher Oermann, MD
Daniel Rosenbluth, MD
Jonathan Wagner, DO
Matthew McLaughlin, MD, MS
Eric Rush, MD, FAAP, FACMG
Rosemary Britts
Sherry Graf, RN, BSN
Francis Cole, MD

Council Members Absent:

Jonathan Cooper, PhD
Michael Burke

MO HealthNet Staff Present:

Joshua Moore, PharmD, Director of Pharmacy
Mark Roaseau, R.Ph, Clinical Pharmacist
Angela Wilson, Pharmacy Operations Manager
Olivia Rush, PharmD, Program Integrity Pharmacist
Elizabeth Sissom, RN, Clinical Management
Lisa E. Smith, Program Development Specialist
Carmen Burton, Administrative Assistant
Timothy Kling, MD, Acting Medical Director
Elizabeth Short, Program Development Specialist
Racheal Rachau, Benefit Program Senior Specialist

Contractors Present:

Blake Shrout, PharmD, AAHIVP, Conduent
Jennifer Colozza, PharmD, Conduent
Luke Boehmer, PharmD, Conduent
Mary-Beth Plum, PharmD, Conduent
Megan Fast, PharmD, Conduent
Paul Fung, PharmD, Conduent
Serena Barden, PharmD, BCPS, Conduent
Chelsea Pendleton, RN, BSN, Wipro
Geri Roling, RN, Wipro
Valerie Schmitz, RN, BSN, Wipro
Ashley Lytton, RN, BSN, Wipro

Others Present:

Anabelle Keohane	Donald Nopper	Jones Guest	Philip Masaitis	Susan Abdel- Rahman
Bill Eicholzer	Hamir Sampat	Kelly Broderick	Rick Kegler	Tom Cogan
Brent Fushimi	Jessica Petrie	Kelly McNeil-Posey	Sean Jones	
Camille Kerr	John Bullard	Lisa	Stormy Cameron	

Welcome, Announcements and Introductions	Patricia Dickson, Council Chair**,** called the meeting to order. Josh Moore, MHD Pharmacy Director, introduced himself and facilitated the meeting on behalf of the MHD.
Minutes Review	**Discussion:** Minutes were reviewed from the November meeting. **Decision:** The Council voted to accept these approved minutes with no revisions.
Pharmacy Program and Budget Update	Elizabeth Short, Josh Moore, Elizabeth Sissom, Christopher Oermann and Claire Elson presented a brief power point of the Pharmacy Program and Budget Updates. Information presented included: - July-Dec 2021 Eligibles by Group - July-Dec 2021 Expenditures by Enrollment Group - July-Dec 2021 Expenditures by Service - FY22 Pharmacy Spend vs July-Dec 2021 Total Medicaid Spend - July 2021- Jan 2022 Pharmacy Expenditures - FY2019 – FYTD2022 Rare Disease Expenditures Per Day - SMART Therapy Provider Outreach - Safe at Home/ DisposeRx Initiative - Diagnosis Codes on Point of Sale Prescription Requirements - CGM Coverage Expansion - Cystic Fibrosis – The Impact of Highly-Effective CFTR Modulatory Therapy on CF Care
Old Business	Josh Moore discussed the Edit Implementation Schedule and the criteria for Previously Approved Clinical Edits, Step Therapies and Prior Authorizations. These handouts were also provided to all attendees and will be posted to the Division's web page: https://dss.mo.gov/mhd/cs/advisory/rdac/meeting.htm

New Business	

Soliris, Ultomiris
 	
C5 Complement Inhibitors Clinical Edit
	Discussion:
	- Josh Moore introduced the products and proposal to the Council for discussion.
	- The Council discussed the recommended criteria for these products.
	  o Discussion arose around opening up the diagnosis of generalized myasthenia gravis (gMG) instead of only documenting a positive anti-acetylcholine receptor (AchR) antibody test.
	  ▪ It was discussed that the medications in this edit have specific FDA approval for use in AchR antibody positive gMG, therefore it was decided our criteria needed to match the drug label.
	  ▪ Potential criteria if indications are expanded outside of AchR antibody positive gMG: Positive AchR antibody test **OR** positive single-fiber EMG study **OR** positive repetitive nerve stimulation study with >10% decrement on the 4th or 5th stimulation (lab dependent).
	  o Discussion arose around the pregnancy considerations for Ultomiris and the lack of these considerations in Soliris.
	  o No recommended criteria changes.
	- Public comments provided by:
	  o Kelly McNeil-Posey with Alexion on Soliris and Ultomiris
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Scenesse Clinical Edit	**Discussion:**
	- Josh Moore introduced the product and proposal to the Council for discussion.
	- The Council discussed the recommended criteria for this product.
	  ○ Discussion arose the need to include likely pathogenic variant verbiage throughout the proposal. See below for recommended criteria changes.
	  ○ Discussion arose around the default approval period only being one month and the potential need to increase the approval period as that would only allow for one implant prior to the need of another approval. It was explained that the one month approval would truly apply to the first approval and subsequent approvals would be given for a longer period after a demonstration of benefit.
	  ○ Discussion arose around the dosing limitations of three implants per year. It was explained that the implants should only be needed during the summer, early spring and early fall months in our region, as that is when patients would experience the most diagnosis related issues. Many other states have similar restrictions in place.
	  ○ Recommended criteria changes:
	  ▪ Initial Therapy:
	  • Documented diagnosis of EPP confirmed by:
	  ○ Metal-free protoporphyrin in hemolyzed anticoagulated whole blood (PEE/Porphyrins Evaluation, Whole Blood) AND
	  ○ Genetic testing demonstrating pathogenic **or likely pathogenic variant in FECH gene**
	- No public comment provided.
	
	Decision: The Council voted to accept the recommended criteria with the above revisions in **blue**.

Tavneos Clinical Edit	**Discussion:**
	- Josh Moore introduced the product and proposal to the Council for discussion.
	- The Council discussed the recommended criteria for this product
	  o Discussion arose around the cost vs benefit of product.
	  o No recommended criteria changes.
	- Public comments provided by:
	  o Hamir Sampat with Chemocentryx Inc
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Voxzogo Clinical Edit	**Discussion:**
	- Josh Moore introduced the product and proposal to the Council for discussion.
	- The Council discussed the recommended criteria for this product.
	  o Questions arose around assessment of cardiac function during clinical trials. It was explained that cardiac function was monitored and documented during the trials.
	  o Questions arose around daily subcutaneous injections and the potential psychosocial impact of injections, especially for the pediatric patients. During the clinical trials, some patients had issues with a larger volume being injected thus injection site rotation was suggested to help decrease the issue. Overall, the psychosocial impact was not documented outside of patient diaries.
	  o Discussion arose around the study results showing statistical vs clinical significance in this patient population. Questions arose the helpfulness of this medication in quality of life aspects. It was explained the clinical trials were performed on younger individuals so it's hard to determine at this point as follow up trials are needed to determine significance.
	  o Questions arose on the continuation of therapy criteria of an increase AGV of 1cm. It was explained this criteria was based off of the clinical trial experience.
	  o Questions arose around sleep apnea assessments during the clinical trial and it was discussed that those forms of assessments were completed.
	  o No recommended criteria changes.
	  o No public comments provided
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Other Business	Hemophilia gene therapy expected by the end of the calendar year. Clarification was provided on Over the Counter medications and MHD's 90 day supply requirements.
Closing	Sherry Graf motioned for the meeting to be closed (see attached roll call).

NEXT MEETING: Tuesday, May 10, 2022
615 Howerton Court, Conference Room 202
Jefferson City, MO 65109 or WebEx

Roll Call for February 8, 2022							
Council Member	**November 2021 Minutes**	**C5 Complement Inhibitors Clinical Edit**	**Scenesse Clinical Edit**	**Tavneos Clinical Edit**	**Voxzogo Clinical Edit**	**Closing**	**Adjournment**
Patricia Dickson	Y	Y	Y	Y	AB	Y	Y
Sherry Graf	Y	Y	Y	Y	SY	MY	SY
Michael Burke	A	A	A	A	A	A	A
Jonathan Cooper	A	A	A	A	A	A	A
Gerald Wyckoff	AB	MY	MY	A	A	A	A
Francis Cole	A	SY	SY	Y	Y	SY	Y
Claire Elson	Y	Y	Y	Y	Y	Y	Y
Christopher Oermann	MY	Y	Y	SY	MY	Y	Y
Daniel Rosenbluth	Y	Y	Y	AB	Y	Y	Y
Jonathan Wagner	Y	Y	Y	MY	Y	Y	Y
Matthew McLaughlin	Y	Y	Y	Y	Y	Y	Y
Eric Rush	SY	AB	Y	Y	AB	Y	Y
Rosemary Britts	Y	Y	Y	Y	Y	Y	MY

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