Advisory Council on Rare Diseases and Personalized Medicine

Date	Time	Location
August 9, 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
Christopher Oermann, MD
Matthew McLaughlin, MD, MS
Eric Rush, MD, FAAP, FACMG
Jonathan Cooper, PhD
Rosemary Britts
Daniel Rosenbluth, MD
Jonathan Wagner, DO
Sherry Graf, RN, BSN
Claire Elson, PharmD
Francis Cole, MD

Council Members Absent:

Michael Burke

MO HealthNet Staff Present:

Joshua Moore, PharmD, Director of Pharmacy
Mark Roaseau, R.Ph, Clinical Pharmacist
Angela Wilson, Pharmacy Operations Manager
Elizabeth Sissom, RN, Clinical Management
Lisa Smith, RFP Coordinator
Carmen Burton, Administrative Assistant
Elizabeth Short, Program Development Specialist
Connie Sutter, Fiscal Manager
Kathy Heriford, Benefit Program Senior Specialist
Nikki Ashley, Pharmacy Program Specialist
Hannah Weaver – Pharmacy Student

Contractors Present:

Amanda Williams, PharmD, RPh, Gainwell Technologies
April Ash, PharmD, Conduent
Blake Shrout, PharmD, AAHIVP, Conduent
Chelsea Pendleton, RN, BSN, Wipro
Geri Roling, RN, Wipro
Jennifer Colozza, PharmD, Conduent
Karen Powell, PharmD, MS, Gainwell Technologies
Megan Fast, PharmD, Conduent
Sandy Kapur, PharmD, Gainwell Technologies
Serena Barden, PharmD, BCPS, Gainwell Technologies
Vicki Revel, PharmD, Gainwell Technologies

Others Present:

Faizan Sattar
Gary Gottesman
Jeff Knappen
Jeff Osmundson
Jenna Doerr
Julie Lair
Matt Bradley
Megan Bell
Ray Kong
Susan Rahman
Tyler Whisman

Welcome, Announcements and Introductions	Patricia Dickson, Council Chair**,** called the meeting to order. Josh Moore, MHD Pharmacy Director, introduced himself along with Hannah Weaver, the MHD pharmacy student and facilitated the meeting on behalf of the MHD.
Minutes Review	**Discussion:** Minutes were reviewed from the May meeting. **Decision:** The Council voted to accept these approved minutes with no revisions.

Pharmacy Program and Budget Update	Elizabeth Short and Josh Moore presented a brief power point of the Pharmacy Program and Budget Updates. Information presented included:
	
	- July 2021 - June 2022 Eligibles by Group
	- July 2021 - June 2022 Expenditures by Enrollment Group
	- July 2021 - June 2022 Expenditures by Service
	- FY22 Pharmacy Spend vs Total Medicaid Spend
	- Oct 2021 - June 2022 Medicaid Expansion Participants, Expenditures, and Claim Count –
	  o Discussion arose around the current rate of growth. This is to be reviewed by the FSD enrollment group at the 8/10/22 Oversight Committee Meeting.
	- July 2021 – June 2022 Pharmacy Expenditures
	- FY2019 – FYTD2023 Rare Disease Expenditures Per Day
	  o Additional discussions arose around:
	  ▪ CAR-T reimbursement and the potential of these treatments to be major drivers of future rare disease spend.
	  ▪ Factors contributing to the increase of per participant and per claim expenditure between Oct 2021 and June 2022. Additional research would be necessary but it is not believed that the use of rare disease agents in the expansion population is the sole contributing factor. The increase in expenditure may be due to the use of high-cost medications (i.e., Biktarvy, Humira, etc.)
	- Trikafta Expenditures FY 2022
	  o Due to a potential upcoming label expansion down to 2 years of age the presentation/data may still serve as informative information. No off-label requests have been noted in the MO Medicaid population.

Old Business	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: https://dss.mo.gov/mhd/cs/advisory/rdac/meeting.htm
New Business	

Amvuttra – Transthyretin-Mediated Amyloidosis (ATTR) 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 preferential selection of an agent(s) based on clinical and financial considerations. It was determined that the selection of agents be left up to prescriber discretion.
	  o No recommended criteria changes.
	- No public comment provided.
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Crysvita 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:
	  ▪ Updates made prior to the meeting (requests for participants with baseline fasting serum phosphorus levels in the normal range will be referred to clinical review; continuation of therapy in participants 18 years or older with normal baseline phosphorus levels will require documentation of benefit of therapy).
	  ▪ Continuation of therapy criteria "recent 25-hydroxy vitamin D levels"
	  - Classification of "recent"
	  - Purpose of vitamin D level assessment for continuation of therapy. Supplementation with vitamin D may be necessary for 25-hydroxy vitamin D levels to remain in the normal range for age. It was decided to rephrase the criteria as "recent <u>normal</u> 25-hydroxy vitamin D levels".
	  - Natural (cholecalciferol) versus synthetic (calcitriol) forms of vitamin D.
	- Public comments provided by Dr. Gottesman
	  o Provided rationale for the requested criteria changes that took place prior to the meeting (see above).
	  o Discussed the starting/maintenance doses utilized in clinical practice for patients with normal phosphorus levels and potential for changes in adult dosing frequency based on ongoing research/studies.
	  o Addressed questions regarding the potential for future FDA reviews based on ongoing research.
	
	Decision: The Council voted to accept the recommended criteria with the following changes:
	- Addition of "Requests for participants with baseline fasting serum phosphorus levels in the normal range will be referred to clinical review" initial criteria
	- Addition of <u>normal</u> to continuation of therapy criteria to read "recent normal 25-hydroxy vitamin D levels"
	- Addition of "for participants aged ≥ 18 years & with normal baseline phosphorus levels: documentation of benefit of therapy (examples include but are not limited to: maintenance of reduced pain complaints, improved mobility, stamina, or improving rickets on radiographic evaluation when compared to baseline)" continuation of therapy criteria

Vijoice 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:
	  ▪ How to determine product efficacy if measurement requirements are removed from criteria.
	  ▪ Rate of efficacy as determined by the clinical trial and potential cost impact.
	  ▪ Challenges of diagnosing/treating *PIK3CA*-Related Overgrowth Spectrum (PROS).
	- Public comments provided by Tyler Whisman, Novartis Medical Affairs Oncology pharmacist
	  o Requested the removal of baseline imaging and testing criteria as these were not required for clinical trial inclusion, not required by the National Institutes of Health (NIH) Workshop Diagnostic guidelines, and can increase stress on the patient/caregiver/system
	  o Discussed improvement in patient quality of life/clinical benefit regardless of lesion size.
	  o Requested that there be no potential for dose interruption for participants requiring dose adjustments.
	  o Upcoming EPIK-P2 study.
	  o Prior authorization approval time for dose adjustments/product changes
	
	Decision: The Council voted to accept the recommended criteria with the following proposed change:
	- Added “Participants who are unable to complete baseline imaging must have at least one quantifiable target lesion able to be measured, requests will be subject to clinical review” indented under the initial therapy criteria “documentation of at least one target lesion identified on imaging with baseline measurement of target lesion volume”

Motion to Close	Matthew McLaughlin motioned for the meeting to be closed (see attached roll call).

Other Business/ Rare Disease Topic of Discussion	Potential topics for MHD or the Council to present at future meetings: - Sickle cell annually - DMD – Dr. McLaughlin - Indications for whole exome mapping in November – Dr. Rush Dr. McLaughlin presented on spinal muscular atrophy (SMA). Discussion arose around the outlook of future meetings and a continued hybrid approach was agreed upon.

NEXT MEETING: Tuesday, November 8, 2022; 615 Howerton Court, Conference Room 202 Jefferson City, MO 65109 or WebEx

Roll Call for August 9, 2022						
Council Member	**May 2022 Minutes**	**ATTR Clinical Edit**	**Crysvita Clinical Edit**	**Vijoice Clinical Edit**	**Closing**	**Adjournment**
Patricia Dickson	Y	Y	Y	Y	Y	Y
Sherry Graf	AB	Y	SY	Y	Y	Y
Michael Burke	A	A	A	A	A	A
Jonathan Cooper	Y	Y	Y	Y	Y	Y
Gerald Wyckoff	SY	SY	MY	MY	Y	Y
Francis Cole	MY	Y	Y	SY	Y	MY
Claire Elson	AB	Y	Y	Y	Y	Y
Christopher Oermann	Y	MY	Y	Y	SY	Y
Daniel Rosenbluth	AB	Y	Y	Y	Y	Y
Jonathan Wagner	AB	Y	Y	Y	Y	Y
Matthew McLaughlin	Y	Y	Y	Y	MY	Y
Eric Rush	Y	Y	AB	Y	Y	Y
Rosemary Britts	Y	Y	Y	Y	Y	SY

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