RARE DISEASE ADVISORY COUNCIL MEETING
May 11, 2021
MO HealthNet
VIA WEBEX ONLY

Council Members Present:

Patricia Dickson, MD, Chair
Jonathan Cooper, PhD
Gerald Wyckoff, PhD
Jonathan Wagner, DO
Rosemary Britts
Claire Elson, PharmD
Sherry Graf, RN
Daniel Rosenbluth, MD
Eric Rush, MD, FAAP, FACMG
Christopher Oermann, MD

Council Members Absent:

Matthew McLaughlin, MD, MS
Francis Cole, MD
Michael Burke

MO HealthNet Staff Present:

Joshua Moore, PharmD, Director of Pharmacy
Elizabeth Sissom, RN, Clinical Management
Lisa E. Smith, Program Development Specialist
Timothy Kling, MD, Acting Medical Director
Connie Sutter, Fiscal Manager
Keri Ballew, Drug Rebate Medicaid Specialist
Jackie Hickman, Drug Rebate Unit Supervisor
Olivia Rush, PharmD, Program Integrity Pharmacist
Elizabeth Short, Program Development Specialist
Ambra Stotler, CPhT, Benefit Program Senior Specialist

Contractors in Attendance:

Chelsea Pendleton, RN, BSN, Wipro
Geri Roling, RN, Wipro
April Ash, PharmD, Conduent
Blake Shrout, PharmD, AAHIVP, Conduent
Jennifer Colozza, PharmD, Conduent
Karen Powell, PharmD, Conduent
Luke Boehmer, PharmD, Conduent
Megan Fast, PharmD, Conduent
Sandra Kapur, PharmD, Conduent
Serena Barden, PharmD, BCPS, Conduent

Others Attending:

Albin Karimattam
Bill Eicholzer
Bob Firnberg
Brent Young
Burt519
Dana Pipkin
David Condrick
Emma Selm-Keck
Gina Heinen
James Baumann
Jeff Osmundson
Jessica Petrie
Jonathan Leesman
Kathrin Kucharski
Kelly Maynard
Matthew Bradley
Nicole Linn
Rob Kilo
Shauna Williams
Sue Rahman
Susie Moroney

Welcome, Announcements and Introductions	Patricia Dickson, Council Chair**,** called the meeting to order. Joshua Moore, MHD Director of Pharmacy, introduced himself and facilitated the meeting on behalf of the MHD.
Minutes Review	**Discussion:** Minutes were reviewed from the February meeting. **Decision:** The Council voted to accept these approved minutes with no revisions.
Pharmacy Program and Budget Update	Elizabeth Short and Joshua Moore presented a brief power point of the Pharmacy Program and Budget Updates. Information presented included: - March 2021 Eligibles by Group - July 2020 – March2021 Expenditures by Enrollment Group - July 2020 – March2021 Expenditures by Service - FY21 Pharmacy Spend vs July 2020 – March2021 Total Medicaid Spend - July 2020 – April 2021 Rare Disease Expenditures - FY2019 – FYTD2021 Rare Disease Expenditures - Hepatitis C Elimination Plan - Omnipod Coverage - High Cost Pipeline Update
Old Business	Joshua 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	

Abecma, Breyanzi – CAR-T Cell Therapy Clinical Edit	**Discussion:**
	- Joshua Moore introduced the products for discussion to the Council.
	- The Council discussed the recommended criteria for these products.
	  ○ Discussion arose around removing the trial and failure of Breyanzi for Kymriah and Yescarta until more real world information could be gathered
	  ▪ Not all treatment centers have access to each CAR-T therapy
	  ▪ Removal would allow the treatment centers to better select the appropriate agent for the participant
	  • Cost of side effect treatment (i.e., CRS, neurological toxicities) could outweigh cost of higher cost therapies
	  ○ Questions arose around side effects and the comparisons table between the therapies – was the incidence of AE's in the clinical trials appropriate and expected? How does it compare to real world evidence? – it was explained that less side effects were seen in real world data.
	  ○ Recommended criteria changes:
	  ▪ **For Kymriah:**
	  • ~~**AND participant is not an appropriate candidate for Breyanzi therapy**~~
	  ▪ **For Yescarta:**
	  • ~~**AND participant is not an appropriate candidate for Breyanzi therapy**~~
	- Public comments provided by
	  ○ Susie Moroney with Novartis on Kymriah
	
	Decision: The Council voted to accept the recommended criteria with the above revisions in **blue**.

Amondys 45 – DMD Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Council.
	- The Council discussed the recommended criteria for this product.
	  o Discussion arose around the efficacy data in the clinical trial – it was explained that the DMD therapies were given conditional approval and are further being studied in confirmatory trials.
	  o Questions arose around the Amondys 45 patient population – if a patient had advanced disease would the therapy be beneficial? – it was explained that the renewal criteria for these agents is based on documentation of clinical benefit.
	  o No recommended criteria changes.
	- Public comments provided:
	  o Kathrin Kucharski with Sarepta on Amondys 45
	  o Kelly Maynard, patient advocate
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Evkeeza – HoFH PDL Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Council.
	- The Council discussed the recommended criteria for this product.
	  o No questions arose around the topic.
	  o No recommended criteria changes.
	- No public comments provided
	
	Decision: The Council voted to accept the recommended criteria with no changes.

Imcivree Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Council.
	- The Council discussed the recommended criteria for this product.
	  ○ Questions arose around removing all genetic testing criteria or only the variant of uncertain significance criteria.
	  ▪ Concern of providers using therapy off-label for obesity if all genetic testing criteria was removed.
	  ○ Recommended criteria changes:
	  ▪ **Initial Therapy**
	  • Documentation of genetic testing demonstrating that the variants in *POMC*, *PCSK1*, or *LEPR* genes are interpreted as pathogenic, **or** likely pathogenic, ~~**or VUS**~~
	- No public comments provided
	
	Decision: The Council voted to accept the recommended criteria with the above revisions in **blue**.

Nulibry Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Council.
	- The Council discussed the recommended criteria for this product.
	  ○ Questions arose around the genetic testing cost for those participants still in the hospital at three months – would the hospital cover those costs? – it was explained that the coverage was all situational.
	  ○ Questions arose around efforts to develop newborn screening for this as this disease state is difficult to identify – it was explained no efforts are being explored at this time.
	  ○ Questions arose around the patient population of this therapy – it was explained it is only FDA approved for type MoCD A.
	  ○ Recommended criteria changes:
	  ▪ **Continuation of Therapy:**
	  • Genetic testing to confirm **biallelic** pathogenic variant of *MOCS1* gene
	- No public comments provided
	
	Decision: The Council voted to accept the recommended criteria with the above revisions in **blue**.

Zokinvy Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Council.
	- The Council discussed the recommended criteria for this product.
	  ○ Discussion arose on the large number of clinical trials participants for this disease state.
	  ○ No other questions arose.
	  ○ Recommended criteria changes:
	  ▪ **Approval Criteria:**
	  • Documented diagnosis of HGPS or processing-deficient PLs with either heterozygous *LMNA* **pathogenic or likely pathogenic variant** with progerin-like protein accumulation or homozygous or compound heterozygous *ZMPSTE24* **pathogenic or likely pathogenic variant**
	- No public comments provided
	
	Decision: The Council voted to accept the recommended criteria with the above revisions in **blue**.

Other Business	No other rare disease topics of discussion were presented to the Council.
Closing	Eric Rush motioned for the meeting to be closed (see attached roll call). The next meeting of the Rare Disease Advisory Council is scheduled via WebEx only on Tuesday, August 10, 2021.

Roll Call for May 11, 2021									
Council Member	**February 2021 Minutes**	**CAR-T Cell Therapy Clinical Edit**	**DMD Clinical Edit**	**HoFH PDL Edit**	**Imcivree Clinical Edit**	**Nulibry Clinical Edit**	**Zokinvy Clinical Edit**	**Closing**	**Adjournment**
Patricia Dickson	Y	Y	Y	Y	Y	Y	Y	Y	SY
Sherry Graf	Y	Y	Y	MY	Y	Y	Y	Y	Y
Michael Burke	A	A	A	A	A	A	A	A	A
Jonathan Cooper	Y	Y	SY	Y	Y	Y	Y	Y	Y
Gerald Wyckoff	A	MY	MY	SY	SY	Y	Y	SY	Y
Francis Cole	A	A	A	A	A	A	A	A	A
Claire Elson	Y	Y	Y	Y	Y	Y	Y	Y	Y
Christopher Oermann	A	A	A	Y	Y	Y	MY	Y	MY
Daniel Rosenbluth	Y	Y	Y	Y	Y	SY	Y	Y	Y
Jonathan Wagner	Y	Y	Y	Y	A	A	A	A	A
Matthew McLaughlin	A	A	A	A	A	A	A	A	A
Eric Rush	MY	Y	Y	Y	MY	Y	SY	MY	Y
Rosemary Britts	SY	SY	Y	Y	Y	MY	Y	Y	Y

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