RARE DISEASE ADVISORY COUNCIL MEETING
November 10, 2020
MO HealthNet
VIA WEBEX ONLY

Committee Members Present

Patricia Dickson, MD, Chair
Jonathan Cooper, PhD
Gerald Wyckoff
Francis Cole
Christopher Oermann, MD
Jonathan Wagner
Matthew McLaughlin, MD, MS
Rosemary Britts
Michael Burke
Claire Elson, PharmD
Eric Rush, MD
Sherry Graf

Committee Members Absent

Daniel Rosenbluth

Contractors in Attendance:

Jennifer Colozza, PharmD, Conduent
Megan Fast, PharmD, Conduent
Karen Powell, PharmD, Conduent
Janelle Sheen, PharmD, Conduent
Caleb Forrest, Conduent

MO HealthNet Staff Present:

Joshua Moore, PharmD, Director of Pharmacy
Mark Roaseau, R.Ph, Clinical Pharmacist
Elizabeth Sissom, RN, Clinical Management
Angela Wilson, Pharmacy Operations Manager
Lisa E. Smith, Program Development Specialist
Dr. Timothy Kling, MD, Acting Medical Director
Elizabeth Short, Program Development Specialist
Connie Sutter, Fiscal Manager
Dr. John Dane, State Dental Director
Keri Ballew, Medicaid Specialist
Olivia Rush, PharmD, Program Integrity Pharmacist

Others Attending:

Adam Kopp
Ann Modrcin, MD, MBA
Bethany Zanrucha
Brent Young
Britt Ward
Bryan Moore
Chris Coleman
Chris Guenther
Dana Pipkin
Emily Cooper
Garth Wright
Gina Heinen
Jack Ratchford
Jeff Knappen
Jessica Petrie
Joe Payne
Jonathan Leesman
Julie Dibaise
Karen Floeder
Kathrin Kucharski
Kelly
Kyle
Lucy Hernandez
Matthew Bradley
Maureen
Melissa Basil
Paul
Rick Kegler
Rob Kilo
Sue Rahman
Suzanna Morgan
Tami Sova
Tina Rhinehart
WITTEKI1

Welcome, Introductions and Opening Remarks	Patricia Dickson, Board Chair**,** called the meeting to order. Joshua Moore introduced himself and welcomed the new additions to the meeting, which included Olivia Rush, Program Integrity Pharmacist for MHD and April Ash, PDL Program Manager for Conduent.
Minutes Review	**Discussion:** Minutes were reviewed from the August meeting. Minute revisions included updating the spelling of 'Jonathan' and 'Wyckoff' in addition to Clair Elson being added to the 'Committee Members Present' section. **Decision:** The committee voted to accept these approved minutes with the above 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: - Sept 2020 Eligibles by Group - July – Sept 2020 Expenditures by Enrollment Group - July – Sept 2020 Expenditures by Service - FY21 Pharmacy Spend vs July – Sept 2020 Total Medicaid Spend - FY21 July – Oct 2020 Pharmacy Expenditures - FY19 – FYTD2020 Rare Disease Expenditures - July – Oct 2020 Rare Disease Expenditures - FY18 – FYTD21 Tamiflu Comparison - 2019 vs 2020 Influenza Vaccine Comparison - Show Me Vax – Influenza Vaccine Reports - State Fiscal Quarter 2019-2020 Rebate - Show Me Strong Recovery Plan website (https://showmestrong.mo.gov/)
PA Committee and DUR Board Update	Joshua Moore discussed updates from the September Drug PA Committee meeting and October DUR Board meeting which included: - No changes were made on edit recommendations provided by the August Rare Disease Board - Growth Hormone PDL edits have had post implementation changes with provider feedback (i.e., bone age changes; blood glucose requirements)

Old Business	Joshua Moore discussed the Edit Implementation Schedule and the criteria for Previously Approved Clinical Edits, Step Therapies and PA's. - Reminder was provided about the pharmacy system migration on Dec 5th; migration will require the pharmacy call center/help desk to be closed resulting in the lack of prior authorization processing for a brief timeframe; provider notification is expected soon - COVID vaccine planning has started to help with efficient rollout - Potential drop in pharmacy call center/help desk call volume with the implementation of the transparent diagnosis code checks on incoming POS claims; decrease could also be due lack of physician appointments with COVID; we have also seen an increase in enrollment so difficult to determine the exact impact of the change; plan is to revisit in a couple of months
New Business	

Viltepso – Duchenne Muscular Dystrophy (DMD) Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Board.
	- Questions arose around the clinical trial experience and the small patient size; discussed a small patient population in this disease state is expected.
	- The Board discussed the recommended criteria for this product.
	  ○ Recommended criteria changes:
	  ▪ Improvement, stabilization, **or less than expected decline in disease progression** of motor, pulmonary, or cardiac function from baseline (ex: 6MWT, NSAA, Brooke Upper Extremity Scale, FVC, ejection fraction)
	  ▪ Documentation of **appropriate monitoring for renal function** every three months
	  • Want to leave it open for the provider to decide the best and most cost effective way to test for renal function instead of relying on a specific set of criteria (i.e., cystain C levels)
	  ○ Updates for Viltepso included:
	  ▪ Age restrictions, dosing restrictions and step therapy of Viltepso prior to Vyondys
	- Discussions on value base agreements
	- Public comments provided by:

	○ Ann Modcrin; Children's Mercy ○ Kathrin Kucharksi; Sarepta **Decision:** The Board voted to accept the recommended criteria with the above revisions in blue.
Enspryng – Neuromyelitis Optica Spectrum Disorder (NMOSD) Clinical Edit	**Discussion:** - Joshua Moore introduced the product for discussion to the Board. - The Board discussed the recommended criteria for this product. ○ Questions arose in regards to requiring a threshold in the number of baseline acute attacks prior to approval and if this type of restriction would be appropriate; discussion showed the number of acute attacks can vary greatly along with the length of remission periods (average being 7 months) ○ Updates included combining clinical criteria for Enspryng and Uplizna to create a new NMOSD clinical edit. - Public comments provided by: ○ Maureen Meeley; Viela Bio Inc ○ Jack Ratchford; Viela Bio Inc ○ Joe Payne; Viela Bio Inc ○ Kelly; Alexion **Decision:** The Board voted to accept the recommended criteria with no changes.
Evrysdi – Spinal Muscular Atrophy (SMA) Clinical Edit	**Discussion:** - Joshua Moore introduced the product for discussion to the Board. - The Board discussed the recommended criteria for this product. ○ Recommended changes: ▪ Participant **(female of appropriate age)** must utilize concurrent birth control methods during and for 1-month post-treatment ○ Updates included combining clinical criteria for Evrysdi, Spinraza and Zolgensma to create a new SMA clinical edit. - Discussion on appropriateness of concurrent utilization of SMA therapies or use of multiple SMA therapies over the course of a participants lifetime. - No public comment provided.

	Decision: The Board voted to accept the recommended criteria with the above revisions in blue.

Isturisa – Isturisa Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Board.
	- The Board discussed the recommended criteria for this product.
	  o Recommended criteria changes:
	  ▪ Documentation of failed pituitary surgery or **reason pituitary surgery is not an option**
	  • Desire to more closely match the diagnosis listed in the PI in hopes to decrease confusion
	
	Decision: The Board voted to accept the recommended criteria with the above revisions in blue.

Tecartus – CAR T-Cell Therapy Clinical Edit	**Discussion:**
	- Joshua Moore introduced the product for discussion to the Board.
	- The Board discussed the recommended criteria for this product.
	  o Updates for Tecartus included:
	  ▪ Age restrictions, appropriate diagnosis, and step therapy
	  o Overall edit changes included:
	  ▪ Appropriate provider restrictions and pregnancy checks
	- No public comment provided.
	
	Decision: The Board voted to accept the recommended criteria with no changes.

Other Business	No other rare disease topics of discussion were presented to the Board.
Adjourn	Matthew McLaughlin motioned for the meeting to be adjourned (see attached roll call). The next meeting of the Rare Disease Advisory Board is scheduled via WebEx only on Tuesday, February 9, 2021.

Roll Call for November 10, 2020							
Board Member	**August 2020 Minutes**	**Viltepso/ DMD CE**	**Enspryng/ NMOSD CE**	**Evrysdi/ SMA CE**	**Isturisa CE**	**Tecartus/ CAR T-Cell Therapy CE**	**Adjourn**
Patricia Dickson	Y	Y	Y	Y	Y	Y	Y
Sherry Graf	Y	Y	Y	Y	Y	Y	Y
Michael Burke	Y	Y	Y	Y	Y	Y	Y
Jonathan Cooper	Y	Y	Y	Y	Y	Y	Y
Gerald Wyckoff	SY	MY	SY	Y	Y	SY	Y
Francis Cole	MY	Y	MY	SY	MY	Y	Y
Claire Elson	Y	Y	Y	Y	Y	Y	Y
Christopher Oermann	Y	N	Y	Y	SY	MY	SY
Daniel Rosenbluth	A	A	A	A	A	A	A
Jonathan Wagner	Y	Y	Y	Y	Y	Y	Y
Matthew McLaughlin	Y	SY	Y	MY	Y	Y	MY
Eric Rush	Y	Y	Y	Y	Y	Y	Y
Rosemary Britts	Y	Y	Y	Y	Y	Y	Y

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