

House _____ Amendment NO. _____

Offered By

1 AMEND House Committee Substitute for Senate Committee Substitute for Senate Bill No. 718, Page 1,
2 Section A, Line 2, by inserting after all of said section and line the following:

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4 "208.183. 1. There shall be established an "Advisory Council on Rare Diseases and Personalized
5 Medicine" within the MO HealthNet division. The advisory council shall serve as an expert advisory
6 committee to the drug utilization review board, providing necessary consultation to the board when the board
7 makes recommendations or determinations regarding beneficiary access to drugs or biological products for
8 rare diseases, or when the board itself determines that it lacks the specific scientific, medical, or technical
9 expertise necessary for the proper performance of its responsibilities and such necessary expertise can be
10 provided by experts outside the board. "Beneficiary access", as used in this section, shall mean developing
11 prior authorization and reauthorization criteria for a rare disease drug, including placement on a preferred
12 drug list or a formulary, as well as payment, cost-sharing, drug utilization review, or medication therapy
13 management.

14 2. The advisory council on rare diseases and personalized medicine shall be composed of the
15 following health care professionals, who shall be appointed by the director of the department of social
16 services:

17 (1) Two physicians affiliated with a public school of medicine who are licensed and practicing in
18 this state with experience researching, diagnosing, or treating rare diseases;

19 (2) Two physicians affiliated with private schools of medicine headquartered in this state who are
20 licensed and practicing in this state with experience researching, diagnosing, or treating rare diseases;

21 (3) A physician who holds a doctor of osteopathy degree, who is active in medical practice, and who
22 is affiliated with a school of medicine in this state with experience researching, diagnosing, or treating rare
23 diseases;

24 (4) Two medical researchers from either academic research institutions or medical research
25 organizations in this state who have received federal or foundation grant funding for rare disease research;

26 (5) A registered nurse or advanced practice registered nurse licensed and practicing in this state with
27 experience treating rare diseases;

28 (6) A pharmacist practicing in a hospital in this state which has a designated orphan disease center;

29 (7) A professor employed by a pharmacy program in this state that is fully accredited by the
30 Accreditation Council for Pharmacy Education and who has advanced scientific or medical training in
31 orphan and rare disease treatments;

32 (8) One individual representing the rare disease community or who is living with a rare disease;

33 (9) One member who represents a rare disease foundation;

34 (10) A representative from a rare disease center located within one of the state's comprehensive
35 pediatric hospitals;

36 (11) The chair of the joint committee on the life sciences or the chair's designee; and

37 (12) The chairperson of the drug utilization review board, or the chairperson's designee, who shall
38 serve as an ex officio, nonvoting member of the advisory council.

39 3. The director shall convene the first meeting of the advisory council on rare diseases and

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1 personalized medicine no later than February 28, 2019. Following the first meeting, the advisory council
2 shall meet upon the call of the chairperson of the drug utilization review board or upon the request of a
3 majority of the council members.

4 4. The drug utilization review board, when making recommendations or determinations regarding
5 beneficiary access to drugs and biological products for rare diseases, as defined in the federal Orphan Drug
6 Act of 1983, P.L. 97-414, and drugs and biological products that are approved by the U.S. Food and Drug
7 Administration and within the emerging fields of personalized medicine and noninheritable gene editing
8 therapeutics, shall request and consider information from the advisory council on rare diseases and
9 personalized medicine.

10 5. The drug utilization review board shall seek the input of the advisory council on rare diseases and
11 personalized medicine to address topics for consultation under this section including, but not limited to:

12 (1) Rare diseases;

13 (2) The severity of rare diseases;

14 (3) The unmet medical need associated with rare diseases;

15 (4) The impact of particular coverage, cost-sharing, tiering, utilization management, prior
16 authorization, medication therapy management, or other Medicaid policies on access to rare disease
17 therapies;

18 (5) An assessment of the benefits and risks of therapies to treat rare diseases;

19 (6) The impact of particular coverage, cost-sharing, tiering, utilization management, prior
20 authorization, medication therapy management, or other policies on patients' adherence to the treatment
21 regimen prescribed or otherwise recommended by their physicians;

22 (7) Whether beneficiaries who need treatment from or a consultation with a rare disease specialist
23 have adequate access and, if not, what factors are causing the limited access; and

24 (8) The demographics and the clinical description of patient populations.

25 6. Nothing in this section shall be construed to create a legal right for a consultation on any matter
26 or to require the drug utilization review board to meet with any particular expert or stakeholder.

27 7. Recommendations of the advisory council on rare diseases and personalized medicine on an
28 applicable treatment of a rare disease shall be explained in writing to members of the drug utilization review
29 board during public hearings.

30 8. For purposes of this section, a "rare disease drug" shall mean a drug used to treat a rare medical
31 condition, defined as any disease or condition that affects fewer than two hundred thousand persons in the
32 United States, such as cystic fibrosis, hemophilia, and multiple myeloma.

33 9. All members of the advisory council on rare diseases and personalized medicine shall annually
34 sign a conflict of interest statement revealing economic or other relationships with entities that could
35 influence a member's decisions, and at least twenty percent of the advisory council members shall not have a
36 conflict of interest with respect to any insurer, pharmaceutical benefits manager, or pharmaceutical
37 manufacturer."; and

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39 Further amend said bill by amending the title, enacting clause, and intersectional references accordingly.