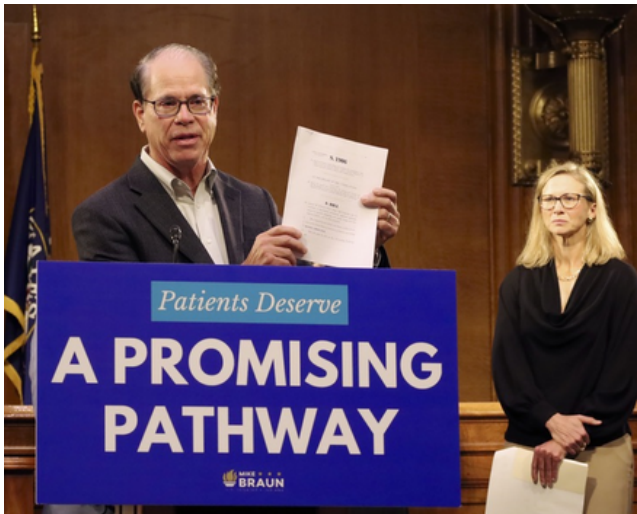


Promising Pathway Act (PPA) 2.0

Introduced by Sen. Mike Braun and Sen. Kirsten Gillibrand

For those living with life-threatening rare and progressive diseases, timely access to treatments can mean the difference between life and death. U.S. Food and Drug Administration (FDA) drug approval pathways unfortunately do not always deliver treatments to patients when there are no meaningful on-label treatments available.



Drugs going through FDA's fastest drug approval pathway take an average of six years before they are approved—and even longer before patients can access them. Very few of those diagnosed with ALS, for instance, live beyond five years. Many of the 10,000 known rare and progressive diseases also substantially reduce lifespan.

What does PPA do?

Access

PPA would allow the FDA to grant time-limited conditional approval for drugs intended only to treat rare, progressive, and congenital diseases that have demonstrated evidence of safety and promising early evidence of effectiveness.

Patients would be able to access these conditionally approved drugs through their insurance.

Sponsors must bring conditionally approved drugs to market within a reasonable time frame.

Guardrails

- FDA has the authority to remove unsafe or ineffective drugs from the market.
- To retain conditional approval beyond 2 years, sponsor must provide additional evidence of effectiveness.
- Drugs may only have Conditional approval for 8 years max.
- Patients must participate in a registry during treatment.
- Patients must provide informed consent.
- Drug is automatically removed from the market if it cannot eventually attain full approval.



Endorsements:

1. I AM ALS
2. The Cure Starts Now
3. Aidan's Avengers
4. A Cure in Sight Foundation
5. Acid Maltase Deficiency Association
6. Alliance to Cure Cavernous Malformation
7. Americans for Prosperity
8. Amyloidosis Support Groups
9. Angelman Syndrome Foundation
10. Anna's Bake Sale Foundation
11. APS Foundation of America
12. Ara Parseghian Medical Research Fund
13. Aubreigh's Army Foundation 328
14. Benny's World
15. Best Day Ever Foundation
16. Beyond Batten Disease
17. CARES Foundation
18. Chelsea's Hope
19. Chondrosarcoma Foundation
20. Choose Joy for Melina
21. CLS Foundation
22. Combined Brain
23. Cure HHT
24. Cure LBSL
25. Cure VCP Disease
26. CURED Foundation
27. Dana's Angels Research Trust
28. Defeat MSA Alliance
29. DMG Collaborative
30. Dravet Syndrome Foundation
31. EHE Foundation
32. End Brain Cancer Initiative
33. Fabry Support & Information Group
34. Foundation for Peripheral Neuropathy
35. Foundation to Eradicate Duchenne
36. Glut1 Deficiency Foundation
37. Grant's Giants
38. Hereditary Angioedema Association
39. Hide and Seek Foundation
40. Hispanic Society for Rare Diseases
41. Histiocytosis Association
42. Hop On A Cure
43. Hope For Hypothalamic Hamartomas
44. Hope for PDCD
45. Jeffrey Modell Foundation
46. Jeffrey Thomas Hayden Foundation
47. Kennedy's Disease Association
48. KIFIA Foundation
49. Koolen-de Vries Syndrome Foundation
50. La Jolla Labs
51. Lauren's Fight for Cure
52. Lennox-Gastaut Syndrome Foundation
53. Li-Fraumeni Syndrome Association
54. MED13L Foundation
55. Musella Foundation for Brain Tumor Research & Information
56. My Kool Brother Foundation
57. National Adrenal Disease Foundation
58. National Association for Continence
59. National Blood Clot Alliance
60. National Fabry Disease Foundation
61. Necrotizing Enterocolitis Society
62. Organic Acidemia Association
63. Patients Rising
64. Periodic Paralysis Association
65. PharmaEssentia
66. Platelet Disorder Support Association
67. Polycystic Kidney Disease Foundation
68. Pompe Alliance
69. Pompe Warrior Foundation
70. Prader-Willi Syndrome Association USA
71. Project Alive
72. Rally for Reid Foundation
73. Reflections of Grace
74. Robert Connor Dawes Foundation
75. RUN DIPG
76. Salla Treatment and Research Foundation
77. Sarcoma Foundation of America
78. SLC6A1 Connect
79. Soleno Therapeutics
80. SSADH Association
81. Stealth Biopharmaceuticals
82. Syngap Research Fund
83. SYNGAP1 Foundation
84. Team Gleason
85. The Autoinflammatory Alliance
86. The Ayla Foundation
87. The Barth Syndrome Foundation
88. The Brooke Healey Foundation
89. The Ehlers-Danlos Society
90. The Foundation for Government Accountability
91. The Foundation to Fight H-ABC
92. The FPIES Foundation
93. The Game On Glio Podcast with Shannon Traphagen
94. The Gold Hope Project
95. The Love4Lucas Foundation
96. The Melina Michelle Edenfield Foundation
97. The Pediatric Brain Tumor Consortium Foundation
98. The Rory Belle Foundation
99. Usher 1F Collaborative
100. Usher Syndrome Coalition
101. Whitley's Wishes, Inc.
102. Yuvaan Tiwari Foundation