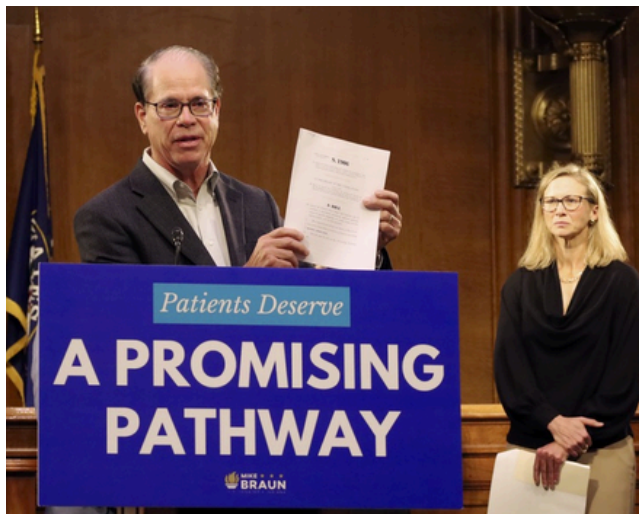


# Promising Pathway Act (PPA) 2.0

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 drugs from the market.
- To retain conditional approval beyond 2 years, sponsor must show 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. A Cure in Sight Foundation
2. Abby's Corner
3. Acid Maltase Deficiency Association
4. ADNP Kids Research Foundation
5. Aidan's Avengers
6. Americans For Prosperity
7. Amyloidosis Support Groups
8. Anna's Bake Sale Foundation
9. Ara Parseghian Medical Research Fund
10. Aubreigh's Army Foundation 328
11. Autoinflammatory Alliance
12. Ayla Foundation
13. Barth Syndrome Foundation
14. Benny's World
15. Best Day Ever Foundation
16. Bonnell Foundation
17. Born A Hero Research Foundation
18. Brooke Healey Foundation
19. Cancer Commons
20. ChadTough Defeat DIPG Foundation
21. Chondrosarcoma Foundation
22. Choose Joy for Melina
23. Cure HHT
24. Cure LBSL
25. Cure Starts Now
26. Cure VCP Disease
27. CURED Foundation
28. Dana's Angels Research Trust
29. Defeat MSA Alliance
30. Defeat MSA Awareness Shoe
31. DMG Collaborative
32. EHE Foundation
33. Ehlers-Danlos Society
34. End Brain Cancer Initiative
35. Foundation for Government Accountability
36. Foundation to Eradicate Duchenne
37. Foundation to Fight H-ABC
38. FPIES Foundation
39. Global Foundation for Peroxisomal Disorders
40. Glut1 Deficiency Foundation
41. Gold Hope Project
42. Grant's Giants
43. Head for Cure
44. Hereditary Angioedema Association
45. Hide and Seek Foundation
46. Histiocytosis Association
47. Hop On A Cure
48. Hope For Hypothalamic Hamartomas
49. Hope for PDCD
50. I AM ALS
51. International Rett Syndrome Foundation
52. Jeffrey Thomas Hayden Foundation
53. Kennedy's Disease Association
54. Kids V. Cancer
55. Kim's Hope
56. Koolen-de Vries Syndrome Foundation
57. Lauren's Fight for Cure
58. Li-Fraumeni Syndrome Association
59. Love4Lucas Foundation
60. Lowe Syndrome Foundation
61. Melina Michelle Edenfield Foundation
62. MSA United Research Consortium
63. Musella Foundation for Brain Tumor Research & Informati
64. My Kool Brother Foundation
65. National Fabry Disease Foundation
66. National Foundation for Ectodermal Dysplasias
67. Necrotizing Enterocolitis Society
68. Neurofibromatosis Network
69. NF2 Biosolutions
70. NW Fare Disease Coalition
71. Organic Acidemia Association
72. Pathways for Rare and Orphan Studies
73. Patients Rising
74. Pediatric Brain Tumor Consortium Foundation
75. Polycystic Kidney Disease Foundation
76. Pompe Alliance
77. Pompe Warrior Foundation
78. Prader-Willi Syndrome Association USA
79. Praxis Precision Medicines
80. Project Alive
81. Project Sebastian
82. PTEN Foundation
83. Rally for Reid Foundation
84. Reflections of Grace
85. Robert Connor Dawes Foundation
86. Rory Belle Foundation
87. RUN DIPG
88. Salla Treatment and Research Foundation
89. SLC6A1 Connect
90. SPATA Foundation
91. SSADH Association
92. Stealth Biopharmaceuticals
93. Storm Heavens
94. Syngap Research Fund
95. SYNGAP1 Foundation
96. T.E.A.M. 4 Travis
97. Team Gleason
98. Usher 1F Collaborative
99. Usher Syndrome Coalition
100. Whitley's Wishes, Inc.
101. Yuvaan Tiwari Foundation