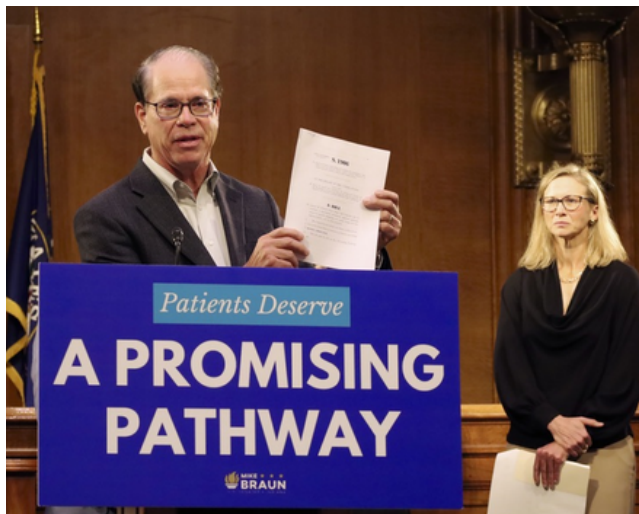


# Promising Pathway Act (PPA) 2.0

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 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. 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. Autoinflammatory Alliance
15. Barth Syndrome Foundation
16. Benny's World
17. Best Day Ever Foundation
18. Beyond Batten Disease
19. Born A Hero Research Foundation
20. Brooke Healey Foundation
21. Cancer Commons
22. CARES Foundation
23. ChadTough Defeat DIPG Foundation
24. Chelsea's Hope
25. Chondrosarcoma Foundation
26. Choose Joy for Melina
27. CLS Foundation
28. Combined Brain
29. Cure HHT
30. Cure LBSL
31. Cure VCP Disease
32. CURED Foundation
33. Dana's Angels Research Trust
34. Defeat MSA Alliance
35. DMG Collaborative
36. Dravet Syndrome Foundation
37. EHE Foundation
38. Ehlers-Danlos Society
39. End Brain Cancer Initiative
40. Fabry Support & Information Group
41. Foundation for Government Accountability
42. Foundation for Peripheral Neuropathy
43. Foundation to Eradicate Duchenne
44. Foundation to Fight H-ABC
45. FPIES Foundation
46. Global Foundation for Peroxisomal Disorders
47. Glut1 Deficiency Foundation
48. Gold Hope Project
49. Grant's Giants
50. Head for the Cure
51. Hereditary Angioedema Association
52. Hide and Seek Foundation
53. Hispanic Society for Rare Diseases
54. Histiocytosis Association
55. Hop On A Cure
56. Hope For Hypothalamic Hamartomas
57. Hope for PDCD
58. I AM ALS
59. Jeffrey Modell Foundation
60. Jeffrey Thomas Hayden Foundation
61. Kennedy's Disease Association
62. Kids V. Cancer
63. KIFIA Foundation
64. Kim's Hope
65. Koolen-de Vries Syndrome Foundation
66. La Jolla Labs
67. Lauren's Fight for Cure
68. Lennox-Gastaut Syndrome Foundation
69. Li-Fraumeni Syndrome Association
70. Love4Lucas Foundation
71. MED13L Foundation
72. Melina Michelle Edenfield Foundation
73. Musella Foundation for Brain Tumor Research & Information
74. My Kool Brother Foundation
75. National Adrenal Disease Foundation
76. National Association for Continence
77. National Blood Clot Alliance
78. National Fabry Disease Foundation
79. National Foundation for Ectodermal Dysplasias (NFED)
80. Necrotizing Enterocolitis Society
81. NW Fare Disease Coalition
82. Organic Acidemia Association
83. Pathways for Rare and Orphan Studies
84. Patients Rising
85. Pediatric Brain Tumor Consortium Foundation
86. Periodic Paralysis Association
87. PharmaEssentia USA
88. Platelet Disorder Support Association
89. Polycystic Kidney Disease Foundation
90. Pompe Alliance
91. Pompe Warrior Foundation
92. Prader-Willi Syndrome Association USA
93. Praxis Precision Medicines
94. Project Alive
95. Project Sebastian
96. PTEN Foundation
97. Rally for Reid Foundation
98. Reflections of Grace
99. Robert Connor Dawes Foundation
100. Rory Belle Foundation
101. RUN DIPG
102. Salla Treatment and Research Foundation
103. Sarcoma Foundation of America
104. SLC6A1 Connect
105. Soleno Therapeutics
106. SPATA Foundation
107. SSADH Association
108. Stealth Biopharmaceuticals
109. Storm the Heavens
110. Syngap Research Fund
111. SYNGAP1 Foundation
112. T.E.A.M. 4 Travis
113. Team Gleason
114. The Ayla Foundation
115. The Bonnell Foundation
116. The Cure Starts Now
117. The Game On Glio Podcast with Shannon Traphagen
118. Usher 1F Collaborative
119. Usher Syndrome Coalition
120. Whitley's Wishes, Inc.
121. Yuvaan Tiwari Foundation