

Promising Pathway Act (PPA) 2.0

June 27, 2024



PKD FOUNDATION
Polycystic kidney disease

A Look at the Next 2 Months

IN-DISTRICT MEETINGS AND LTE PITCHES

1

Draft an LTE for Placement In Local News

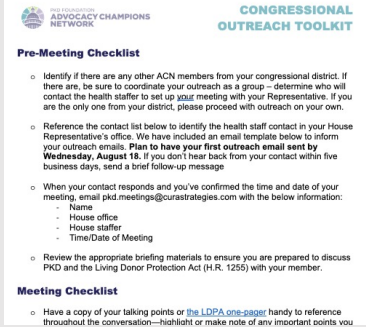
Interested Champions will begin pitching letters to the editor (LTEs) to local news outlets to raise awareness for the LDPA and/or other PKD related issues

2

Schedule an In-District Meeting

Interested Champions will work to secure meetings with their federal lawmakers while they are in the district.

Reference your outreach toolkit for more information!



What is a Letter-to-the-Editor? (LTE)



SHORT AND CONCISE. Typically 250 words or less.



Sometimes written in direct response to an article or op-ed in a publication.



Fast publishing turnaround time.



Perfect way to respond to the issues of the day and publish your views.



In-District Meetings

- Determine which lawmaker you want to meet with and contact your Member's office to schedule in person or virtual meetings **no later than Friday, August 9**
- **Meeting Agenda:**
 - Introductions
 - What is PKD?
 - The Living Donor Protection Act, Living Organ Donor Tax Credit Act, Home Dialysis, etc.
 - Questions?
- **Don't forget to follow up!!**

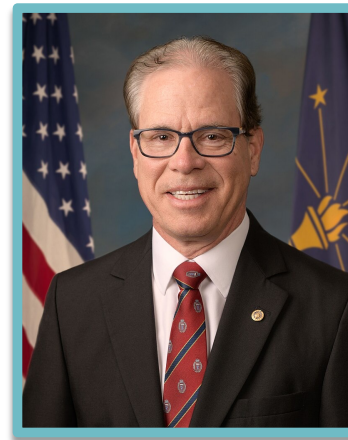
Reminder: Notify CURA staff when your meeting has been secured so that we can participate virtually if applicable.



About the Promising Pathway Act 2.0 (S. 4426)

The Promising Pathway Act (PPA) 2.0 was **introduced on May 23, 2024**, by Sen. Mike Braun and Sen. Kirsten Gillibrand.

This legislation 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.



Sen. Mike Braun
(R-IN)



Sen. Kirsten Gillibrand
(D-NY)



PPA 2.0 Original Sponsors

Senate (10):

- Sen. Mike Braun (R-IN)
- Sen. Kirsten Gillibrand (D-NY)
- Sen. Kevin Cramer (R-ND)
- Sen. Joe Manchin III (D-WV)
- Sen. Eric Schmitt (R-MO)
- Sen. Alex Padilla (D-CA)
- Sen. J.D. Vance (R-OH)
- Sen. Cory Booker (D-NJ)
- Sen. Josh Hawley (R-MO)
- Sen. Peter Welch (D-VT)
- Sen. Lisa Murkowski (R-AK)



The Promising Pathway Act 2.0 is a critical **bipartisan legislative initiative** designed to expedite the approval process for drugs targeting rare and life-threatening diseases.



PPA 2.0 Endorsements

The act has garnered widespread support from over 100 patient advocacy groups and stakeholders, including the **PKDF**, emphasizing the collective effort to increase access to life-saving treatments for rare disease.

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 to Eradicate Duchenne
43. Foundation to Fight H-ABC
44. FPIES Foundation
45. Global Foundation for Peroxisomal Disorders
46. Glut1 Deficiency Foundation
47. Gold Hope Project
48. Grant's Giants
49. Head for the Cure
50. Hereditary Angioedema Association
51. Hide and Seek Foundation
52. Hispanic Society for Rare Diseases
53. Histiocytosis Association
54. Hop On A Cure
55. Hope For Hypothalamic Hamartomas
56. Hope for PDCD
57. I AM ALS
58. Jeffrey Modell Foundation
59. Jeffrey Thomas Hayden Foundation
60. Kennedy's Disease Association
61. Kids V. Cancer
62. KIFIA Foundation
63. Kim's Hope
64. Koolen-de Vries Syndrome Foundation
65. La Jolla Labs
66. Lauren's Fight for Cure
67. Lennox-Gastaut Syndrome Foundation
68. Li-Fraumeni Syndrome Association
69. Love4Lucas Foundation
70. MED13L Foundation
71. Melina Michelle Edenfield Foundation
72. Musella Foundation for Brain Tumor Research & Information
73. My Kool Brother Foundation
74. National Adrenal Disease Foundation
75. National Association for Continence
76. National Blood Clot Alliance
77. National Fabry Disease Foundation
78. National Foundation for Ectodermal Dysplasias (NFED)
79. Necrotizing Enterocolitis Society
80. NW Fare Disease Coalition
81. Organic Acidemia Association
82. Pathways for Rare and Orphan Studies
83. Patients Rising
84. Pediatric Brain Tumor Consortium Foundation
85. Periodic Paralysis Association
86. PharmaEssentia USA
87. Platelet Disorder Support Association
88. Polycystic Kidney Disease Foundation
89. Pompe Alliance
90. Pompe Warrior Foundation
91. Prader-Willi Syndrome Association USA
92. Praxis Precision Medicines
93. Project Alive
94. Project Sebastian
95. PTEN Foundation
96. Rally for Reid Foundation
97. Reflections of Grace
98. Robert Connor Dawes Foundation
99. Rory Belle Foundation
100. RUN DIPG
101. Salla Treatment and Research Foundation
102. Sarcoma Foundation of America
103. SLC6A1 Connect
104. Soleno Therapeutics
105. SPATA Foundation
106. SSADH Association
107. Stealth Biopharmaceuticals
108. Storm the Heavens
109. Syngap Research Fund
110. SYNGAP1 Foundation
111. T.E.A.M. 4 Travis
112. Team Gleason
113. The Ayla Foundation
114. The Bonnell Foundation
115. The Cure Starts Now
116. The Game On Glio Podcast with Shannon Traphagen
117. Usher 1F Collaborative
118. Usher Syndrome Coalition
119. Whitley's Wishes, Inc.
120. Yuvaan Tiwari Foundation

← We are here!



Background



Drugs going through the FDA's fastest drug approval pathway take an **average of six years** before they are approved



The FDA's drug approval process follows several different steps including **discovery and development, preclinical testing, clinical trials, FDA review, and post-market monitoring.**



There are **few or no existing treatments** for many life-threatening diseases, and small patient populations for clinical trials make it difficult to gather sufficient data to meet the FDA's standards.



Bill Overview

Purpose and Goals

- Designed to expedite the approval process for drugs that target rare, progressive and life-threatening diseases by allowing the FDA to grant time-limited conditional approval that have evidence of safety and effectiveness.
- Ensures that patients with urgent needs can access treatments quickly.

Key Guardrails

- Required eligible drugs to have completed Phase 1 trials and show positive results in Phase 2 to be eligible for conditional approval.
- Conditional approval lasts up to two years and can be renewed up to three times.
- FDA has the authority to withdrawal approval for safety concerns or if eligibility criteria is no longer met.

Responsibilities

- Patients must participate in an observational registry during treatment.
- Patients also must provide informed consent.
- Sponsors are responsible for maintaining and submitting patient data to the registry.
- Sponsors must bring conditionally approved drugs to the market within 180 days of approval.

Safety and Accountability

- Labeling and promotional materials will be reviewed by the FDA within 30 days of dissemination.
- A list of conditionally approved drugs will be publicly available on the FDA's website.
- Private and public payers are required to cover conditionally approved drugs for the period of their conditional approval.



Concerns about PPA 2.0

- Early clinical data is not always predictive of efficacy and safety of a drug
- Provisionally approved drugs may make the drugs less accessible due to health insurance coverage and reimbursement challenges



QUESTIONS?



Contact Us



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