ILLINOIS DEPARTMENT OF PUBLIC HEALTH
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Illinois Rare Disease Commission

Monday May 20, 2024
12-1 PM Virtual

Minutes

Name	Present (Y/N)	Role	Affiliation
***Maria "Ria" Pollock**	na	Affected / Caregiver; Advocacy Group	*(Chair)* Living with rare disease
Joyce Clay	X	Affected / Caregiver; Health Professional	Daughter with rare disease
Tim Cunniff	X	Industry	*(Vice Chair)* Paragon Biosciences
Stacey Feuer	na	Affected / Caregiver; Health Professional	Living with rare disease
TaLana Hughes		Affected / Caregiver; Advocacy Group	Sickle Cell Disease Association of Illinois (SCDAI)
Lara Pullen	X	Affected / Caregiver; Advocacy Group; Industry	Chion Foundation / child with rare disease
Stacey Pigott		***Pending***	
Vacant		***Appointed***	
Vacant		***Appointed***	
Vacant		***Appointed***	
Vacant		***Appointed***	
William Hauter	X	*Policymaker (State Representative 87th District)*	Certified in Emergency Medicine
Linda Holmes		Policymaker	Living with chronic illness
Sonya Harper		Policymaker	By Je'Mia Irving
Vacant	-	*Policymaker*	*TBD*

Attendees: Joan Ehrhardt, IDPH Facilitator, Jon Vlasnik PharmD, Alexion, AstraZeneca Rare Disease Ken Ring, Amgen, Kevin Hall, Sanofi, Madison Zeltwanger, Maya Thirkill (Epidemiologist), and National Academies of Sciences, Engineering and Medicine-Associate Program Officer, Mary Ellen Baker, Steve Patterson, and Stacey Repotski, PharmD, Acadia Pharmaceuticals, John Conrad, iBio, Lori Cecutti, Rocket Pharmaceuticals, Brandon Linton, Ben Chandhok.

Welcome and Introductions ***-Tim/Commission Members***

Late Submissions: None

Adoption of Agenda & Approval of Meeting Minutes Approval of the April meeting was tabled due to lack of quorum.

Public Comment: Steve Patterson mentioned that at the Public D&T Meeting the public comment period was moved to the end of the agenda. As a result, there was little time to discuss. He stated that the Public Comment period was treated differently previously, being held earlier in the meeting. That allowed for more discussion, more input, before the Board made decisions about products under review. Tim agreed that is a little different in his experience. Steve mentioned that he has had experience with similar Boards in 10 or 12 states and this was unusual. Tim offered to reach out to that Board and invite them to present at a future meeting of the IRDC. Joan asked for clarification, information about the Board. [Drugs and Therapeutics Advisory Board (https://hfs.illinois.gov/medicalproviders/pharmacy/committeeondrugsandtherapeutics.html)]

Old Business/New Business/Discussion Access to Pharmaceuticals – Strategies for Access

Off-label uses – Tim discussed the strategy for use, approvals, labeling, limitations, and related challenges. Tim shared some specifics around a condition, Tourettes disorder. Access to specific therapeutics can be limited by the specifics of the labeling and clinical trials, being a point of denial by some insurers. Tim said that there is a role for state government to set policy to help make treatments available through expanded insurance coverage. He asked others to discuss.

Lara discussed some particular strategies and challenges that apply to Prader-Willi syndrome. Closing the gap between approval of therapeutics for a specific diagnosis and approval of that therapeutic for similar but not identical diagnosis. Lara shared the example of daytime fatigue-excessive daytime sleepiness vs. narcolepsy, with medication specifically approved for one, but not the other. Possibility or probability of working is high, but patient population has not been specifically tested to show efficacy. As a result, coverage is denied. The gap related to the disconnect between disease, symptomatology, and approvals “is brutal.” In the rare disease community, a high likelihood of efficacy is not enough and the specific rare disorder numbers are so small, there is almost no chance of having a potential treatment tested and approved for any single rare disease. Tim said on the federal level perhaps FDA approvals could be helped by an all-comers policy or basket trial, suggested Lara. There is precedence for this approach. Lara submitted a paper making this argument. It would be great if state could recognize this – create some leniency while FDA is working to address separately. Tim mentioned the policy reviewed by IRDC last month was great, and there are things that states can do to help. Discussion of how to build evidence for safety and efficacy and expand usage on label. Current policy disadvantages conditions that are most fragile, most rare, most complex. Tim and Lara discussed the IRDC role to identify issues and opportunities to bring to policy makers’ attention. Tim suggested the IRDC begin prioritizing issues, strategies, and guidance. Lara asked what would be more likely to move forward?

Stacy Repotski mentioned that the D&T committee is the committee that brings forth prior authorization recommendations for State Medicaid and asked if there is an opportunity for IRDC to help D&T shape prior authorization policies that meet the needs of rare disease patients. Lara asked Tim if a member could be invited to attend IRDC and discuss their work. “Let’s get on the same team.”

Overcoming insurance denials – Tim mentioned that insurance denials come in different stripes. Tim comes back to policy and guidance and believes that more specific strategies are needed. Lara asked if they have rights as patients? Are they written down anywhere? Could that be a resource? Tim said that the policy reviewed last month had specific guidance to protect patients. Lara said wouldn’t it be great to create a patient bill of rights and have it publicly available. Joan shared the link to Illinois Compiled Statutes. [https://www.ilga.gov/legislation/ilcs/ilcs.asp]

Lara asked what is the accountability? Is there any within the system? And if there is, how does the patient access that? Are patient rights aspirational (vs. practical)? Lara mentioned that NORD may have examples of specific language.

Announcements: The next meeting date and topic were provided, being Monday 17 June 2024 and continuing discussion of Access to Pharmaceuticals. Joyce mentioned that she has not been receiving emails. Joan agreed to check contacts after the meeting.

Adjournment: The meeting adjourned a few minutes early ~12:50 pm

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