November Legislative Meeting

November 18th, 2015
Agenda:


2. Reauthorizing the Priority Review Voucher Program – Nancy Goodman, Executive Director, Kids V Cancer

3. Clinical Laboratories Improvement Amendments Modernization – Tara Burke Ph.D., Policy Analyst, Association for Molecular Pathology

4. Capitol Hill Updates – Max Bronstein, Senior Director of Advocacy & Science Policy, EveryLife Foundation for Rare Diseases

5. RDLA updates: RareVoice Recap & Rare Disease Week on Capitol Hill – Andy Russell, Associate Director of Advocacy & Government Relations, EveryLife Foundation for Rare Diseases
H.R. 3742: Access to Marketplace Insurance Act

- Rick Collin, Senior Policy Advisor, Office of Congressman Kevin Cramer
Help Americans with Preexisting Conditions Keep Their Health Plans
Cosponsor H.R.3742 – The Access to Marketplace Insurance Act

Dear Colleague,

The goal of the Affordable Care Act (ACA) was to reduce the number of uninsured Americans and provide certain protections for individuals with preexisting health conditions. We can all agree these are noble goals, but it seems CMS is working against both of them.

For decades, non-profits, civic groups, and places of worship have stepped forward to provide a safety net for the most ailing among us—those with catastrophic, chronic, or rare diseases. These charitable organizations use donations to help pay health insurance premiums and ensure patients do not lose coverage just when critical care is most needed.

Last March, the Centers for Medicaid and Medicare Services (CMS) released an interim final rule which gave authority to insurers offering plans on the Exchanges to deny non-profit charities from providing premium assistance. Since patients with rare diseases and catastrophic illnesses disproportionately use these programs, this rule has the effect of pushing individuals with preexisting conditions off the health plans they purchased in an Exchange. That means fewer insured Americans, and more patients with complex conditions in the federal safety net.
Under the ACA, the Law provides federal subsidies for health insurance. Why, then, did the Administration offer a rule to prevent Americans from doing the same out of charity when they see a need? Since the release of the interim final rule, plans in 22 states have announced a prohibition. The Access to Marketplace Insurance Act, H.R.3742, would remove the handcuffs from private charities, allowing them to assist insured patients who need help paying their premiums. The Law already compels insurance companies to accept premium assistance from the Affordable Care Act, Indian Tribes, and State or federal programs. Individual Americans should have the right to organize and do the same through their charity of choice.

Please consider joining H.R.3742 as a cosponsor and support non-profits as the backbone of the health safety net for our fellow Americans. Contact Rick Collin in my office at Rick.Collin@mail.house.gov, or call 5-2611.
Reauthorizing the Priority Review Voucher Program

- Nancy Goodman, Executive Director, Kids V Cancer
Proposal for Modernization of CLIA Regulations for Laboratory Developed Testing Procedures

Tara Burke Ph.D., Policy Analyst, Association for Molecular Pathology

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Desired Outcomes

- Patients receive the most appropriate test for their clinical condition
- Laboratory developed testing procedures (LDPs) are accurate, precise, clinically relevant, and monitored for continued quality performance
- Health care professionals able to provide professional services to their patients without undue restrictions
- Preserve the ability of the laboratory community to provide surge capacity in public health emergencies
- Preserve patient access to a variety of testing options
- Regulatory oversight does not slow innovation, constrain flexibility and adaptability, or limit a test’s sustainability
The proposal is built on three major pillars:

- Enhancing Transparency
- Ensuring Quality
- Preserving Innovation
## LDPs vs. IVDs

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<tr>
<th>Laboratory Developed Testing Procedures</th>
<th>In Vitro Diagnostics Kits</th>
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<td>Developed by a health care professional, often at the request of your treating physician</td>
<td>Developed by a manufacturer</td>
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<tr>
<td>Conducted within the lab that designed and validated the LDP</td>
<td>Boxed and shipped to external location; Conducted by those not involved in developing the IVD</td>
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<td>LDP evaluated by other third party experts</td>
<td>IVD evaluated by FDA</td>
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<tr>
<td>Continuously monitored</td>
<td>Information not always accessible which prevents third parties from being able to continuously monitor</td>
</tr>
<tr>
<td>Ability to modify as needed in response to new technology or understanding</td>
<td>Manufacturers must again complete FDA review system to modify IVD</td>
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Regardless, the information a patient receives should be verified, accurate, and clinically relevant.
Why modernize CLIA regulations?

• CMS has a long history of holding laboratory practices to certain regulatory standards (i.e. CLIA regulations)
• This process can and should be improved regardless of any other activity on the issue
• The proposal offers a streamlined, cost-effective approach to addressing clinical validity, transparency, and other issues
Proposal Applies to LDPs

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Key Features

• Tiered; risk-based
• Assures both analytical and clinical validity without jeopardizing innovation
• Provides for rapid response during public health emergencies
• Provides transparency so physicians and patients have essential information
• Levels the playing field by applying the same regulatory principles to anyone who develops an LDP
• Provides for pre-introduction review of high & moderate risk LDPs
• Special considerations for LDPs for rare diseases and compassionate use
• Provides for enhanced standards
• Requires proficiency testing or alternative assessment for all LDPs
• Avoids duplication of activities within and between federal agencies

Timeline: 2 years to final rule, another 2 years after final rule to take effect
## LDP Risk Classification

<table>
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<tr>
<th>Classification</th>
<th>Definition</th>
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<td><strong>Low</strong></td>
<td>An LDP for which the laboratory makes no claim that the test result alone determines diagnosis, prognosis or direction of therapy, absent other clinical information or diagnostic procedures, OR; the consequence of an incorrect result or interpretation is unlikely to lead to serious morbidity or mortality, either for the patient or the public health. <strong>LDPs used for rare diseases, for public health emergencies, and for infectious agents that are not serious threats to the public health</strong> are classified as low-risk.</td>
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<tr>
<td><strong>Moderate</strong></td>
<td>Taking medical context into consideration, an LDP that is used to diagnose a disease, predict risk of disease, or risk of progression of a disease, or patient eligibility for a specific therapy to treat a disease, that is associated with significant morbidity or mortality, AND; the test lends itself to inter-laboratory comparisons or proficiency testing.</td>
</tr>
<tr>
<td><strong>High</strong></td>
<td>Taking medical context into consideration, an LDP that is used to diagnose a disease, predict risk of disease, or risk of progression of a disease, that is associated with significant morbidity or mortality, AND; uses methodologies that involve proprietary algorithms or computations such that the test results cannot be tied to the methods used or inter-laboratory comparisons cannot be performed.</td>
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Ensuring that an LDP is accurate and reliable

1. Development: LDP developed; lab classifies based on risk definition
2. Submission: Lab submits High & Moderate Risk LDP information to reviewer before offering the service to the public
3. Review: CMS/Third Party Reviewer reviews LDP to ensure that it is accurate and reliable
   a. Timing based on risk classification
   b. CMS may reclassify risk; lab may appeal
4. Public Database: CMS creates publicly accessible entry for LDP
5. Decision: Review decision issued; lab may appeal
6. Post Introduction Monitoring: Lab participates in proficiency testing and is inspected
7. Adverse Event Reporting: LDPs that pose immediate jeopardy to the laboratory's patients must be reported directly to CMS
8. Modifications: Lab continuously improves LDP over time
   a. Depending on the modification(s), may require a new submission & review of LDP, OR solely updating the existing LDP form
Compassionate Use

A single patient with suspected or established serious or immediately life threatening condition may be offered an LDP that has not been approved as long as the ordering physician has been notified in writing that the LDP has not been approved, and the physician provides the order to proceed. The compassionate use order must be documented by the laboratory.
For more information on AMP’s proposal:

http://amp.org/advocacy/CLIAModernization.cfm
Capitol Hill Update

Max Bronstein, Senior Director, Advocacy & Science Policy
EveryLife Foundation for Rare Diseases
21st Century Cures Initiative

- Bipartisan effort led by Reps. Upton (R-MI) & DeGette (D-CO) (Energy & Commerce Committee) to improve the biomedical ecosystem
- The Committee spent over 1 year gathering input from stakeholders and released 4 draft bills
- The 21st Century Cures Act (HR 6) includes a variety of provisions of critical importance to the rare disease community
- Passed by the House in July 344–77
Whitepaper released in January by Sens. Alexander (R–TN) & Burr (R–NC) – Meetings have been held w/ NIH & FDA

Foundation Action Alert
  • “MAKE INNOVATION FOR HEALTHIER AMERICANS A PRIORITY”

Paying for funding increases will be a major hurdle
  • Budget deal used the payfors from HR 6

HELP Committee promises that a draft will be released any day
  • All “controversial” provisions will be added during mark–up
    • Dem’s want safety & pricing provisions in exchange to those favorable to industry
    • They plan to mark–up the bill before the end of the year
    • Senate floor vote not likely until 2016
    • Then to Conference Committee

Foundation will be activating grassroots to ensure rare disease provisions are included during mark–up
Budget Deal & Sequestration

- The Budget Control Act of 2011 prevented increases in federal spending through arbitrary spending caps.
- Caps have stymied increases for FDA & NIH.
- The new budget deal would raise the debt limit (2 years) and provide room to grow budgets of federal agencies ($80B over two years – substantial, but not a game changer).
- Working the Coalition for Health Funding, ELF has pushed for higher caps and sequestration relief, but still a threat – this deal provides partial relief.
- The Foundation will continue to advocate to end sequestration and fully fund FDA & NIH.
Dr. Robert Califf is President Barack Obama's nominee to lead the Food and Drug Administration.

Yesterday he defended his record as Senators pressed him about rising drug prices, slow approval times for new drugs and medical devices and his ties to the pharmaceutical industry.

The committee wanted to review and question the nominee for FDA commissioner before Cures companion legislation was introduced to keep the focus of the hearing on the potential commissioner and not on the potential Cures legislation.
Questions?

- mbronstein@everylifefoundation.org
RDLA Updates

- RareVoice Awards Gala Recap
- Feb. 29th - Mar. 3rd: Rare Disease Week on Capitol Hill
More than 300 patient advocates and industry leaders as well as staff from Capitol Hill and federal agencies joined us to celebrate advocates who have given rare disease patients a voice on Capitol Hill;

Next year’s RareVoice Awards will be held on November 16th, 2016 at the Arena Stage in Washington, DC

See more photos soon on RDLA’s Facebook page and at RareVoiceAwards.org
Travel Scholarships
- Apply for a Travel Scholarships: **Now until – December 31st**
- Applicants will be notified: **January 4th**

Registration:
- Registration for RDW on Capitol Hill on rareadvocates.org:
  - Opens - **January 4th**
  - Closes - **February 15th**

Informational Webinars
- First Rare Disease Week webinar: **January 20th**
- Second Rare Disease Week webinar: **February 23rd**
February 29th – March 3rd

Registration opens January 4th, 2016 on rareadvocates.org

- Monday, February 29th: Rare Disease Day at the NIH
- Monday, February 29th: Rare Disease Documentary Screening
- Tuesday, March 1st: Legislative Conference at FHI 360
- Wednesday, March 2nd: Lobby Day
- Thursday, March 3rd: Congressional Caucus Briefing
- Thursday, March 3rd: Rare Artist Reception

Cost: FREE to attend for Patient Advocates*
*Travel scholarships are available

To see event pictures from previous years go to: rareadvocates.org/rdw
Stipends are provided through the RareGiving Program which ensure Congress, the FDA and other government agencies have the opportunity to hear directly from patients.

Advocates must attend the Legislative Conference to receive their travel stipend.

Travel stipends will be given out at the Legislative Conference Registration:
- Advocates must present photo ID, provide a Social Security Number
- Stipends can be used for any travel expenses: hotel, food, airfare, gas, parking, etc.

Stipends will range from $300-$1000 maximum, depending upon the distance the recipient has to travel.

Apply online through December 31st, we will grant as many stipends as possible, and notify winners on January 4th.

Only one travel stipend per family.
If you or someone you know with a rare disease cannot make it to Rare Disease Week on Capitol Hill, RDLA still wants your voices to be heard.

Please share your story with the Foundation and we will make sure it is hand delivered to your representative in D.C.

Submit your *Rare* Story here:
http://rareadvocates.org/rdw/patientstories/
Questions?

For all the information listed on this presentation go

RareAdvocates.org

Contact Information:

Andy Russell

Associate Director of Advocacy and Government Relations
EVERYLIFE FOUNDATION FOR RARE DISEASES

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www.EveryLifeFoundation.org www.RareAdvocates.org
Next RDLA Meeting

December 16th, 2 PM EST

Webinar and Conference call

If you would like to add an item to the agenda for the next meeting please send requests to: arussell@everylifefoundation.org