Rare Disease Legislative Advocates
July Legislative Meeting

July 19th, 2017
Health Insurance Markets: What is Happening and How Can Congress Fix It?
CAHC: A DIVERSE COALITION
CHALLENGES FACING THE ACA MARKET
The ACA made massive changes to health markets – some positive and some negative.

- It created new consumer protections, corrected market imbalances, and reduced the number of uninsured Americans to historic lows.
- Yet, overreach by the ACA has also contributed to high and growing health insurance premiums.
- Plan choice and competition have declined.
- ACA’s enrollment risk pools are seriously unbalanced.

**Result:** An unstable and expensive market that is driving away many of the healthy consumers needed to hold coverage costs down.

**Further:** Massive uncertainty created by political turmoil has made a bad situation much worse.
ACA EXCHANGE ENROLLMENT: ROUGHLY 10.3M SIGN-UPS AFTER 2017 OPEN ENROLLMENT

ACA Insurance Exchange Enrollment
2014 - 2017

Open Enrollment Plan Selections
(Sign-Ups Only)

Effectuated Enrollment Total
(Sign-Ups & Paid Premiums)

*Approximate enrollment total.
LOWER ENROLLMENT AMONG YOUNG (TYPICALLY HEALTHIER) ADULTS MEANS RISK IS GREATER, PREMIUMS MORE EXPENSIVE

2013 POTENTIAL EXCHANGE POPULATION VS. 2017 ENROLLED POPULATION, BY AGE

2013 Potential Population
- Under 18: 14%
- 18-34: 36%
- 35-54: 34%
- 55 and Older: 16%

2016 Enrolled Population
- Under 18: 10%
- 18-34: 27%
- 35-54: 36%
- 55 and Older: 27%

Source: CAHC 2016 Exchange Report, 2017 CMS
2017: WHERE WE ARE
Average Second Lowest Cost Silver Plan Premiums for a 27-Year Old (Before Tax Credits), 2014-2017

2014: $218, 2%
2015: $224, 7%
2016: $242, 25%
2017: $302

*Data are for the 39 states utilizing HealthCare.gov only.

### Average National Silver Individual Exchange Coverage Deductibles*

<table>
<thead>
<tr>
<th>Year</th>
<th>Deductible</th>
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<tr>
<td>2014</td>
<td>$2,907</td>
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<td>2015</td>
<td>$2,927</td>
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<tr>
<td>2016</td>
<td>$3,117</td>
</tr>
<tr>
<td>2017</td>
<td>$3,572</td>
</tr>
</tbody>
</table>

* Individual Silver is the most popular Exchange plan.

Source for Individual Market: Data.HealthCare.gov via HealthPocket’s Infostat Reports.
2018:
WHAT WE EXPECT
2018 PLAN OUTLOOK

• Federal filing deadline was June 21
  • Large premium increases mirroring the 2017 spikes
  • Large cost sharing increases limiting access
  • Continual pull out of major carriers in several states – with more likely to come as we get closer to open enrollment

• 44 counties are projected to have no insurers impacting 31,268 people

• 1,200 counties - nearly 40% of counties nationwide – could have only one issuer impacting 2.4 million people

• Market problems amplified by political uncertainty around cost sharing subsidies, other reforms
  • Unclear anything has any chance of passing the Senate
County by County Analysis of Current Projected Insurer Participation in Health Insurance Exchanges
OBAMACARE TO
TRUMPCARE
• AHCA Repeals Some Obamacare Provisions
  • Individual and Employer mandate penalty
  • Taxes

• Four new policy buckets:
  • Revised tax credit based on age and income, not premium
  • Medicaid Reform
  • Insurance Reforms
  • Stabilization policies

• Impact:
  • Lower premiums, deficits
  • More uninsured
For 2018-2019, younger enrollees are receive greater relative subsidies (while still retaining ACA income-based structure) as a mechanism to improve the risk pool.

Starting in 2020, an age-based credit is applied. Older individuals receive greater tax credits (2:1 ratio), with wealthier individuals eligible for credits. Credit is not pegged to premiums.

Impact of the new credit (whether better or worse than ACA) varies by age, income, and geographical location.

Starting in 2020, phase out of Medicaid expansion enrollees begins. Most expansion enrollees are expected to be off Medicaid within 3-5 years.

Starting in 2020, changes the Medicaid financing structure to a per capita caps structure.

Expected to significantly reduce the number of people on Medicaid and the cost of the program.

Most ACA tax credits are rescinded. Repeal of the health insurance, medical devise, and pharmaceutical taxes are expected to lower premiums. Repeal of the Cadillac tax removes upward pressure on cost sharing in employer sponsored plans.

Zeros out the penalty for both the employer and the individual mandate but does not repeal them.

Employer reporting requirements are still in effect.

Individual mandate is replaced with a 30% premium surcharge on premiums for not maintaining continuous coverage. Expected to be less effective than the mandate for risk pool health.
POLICY MAKERS MUST ACT NOW

1. Stabilize markets
   • Premium stabilization and cost sharing reductions are key
   • Repeal taxes that directly impact premiums (e.g., tax on health insurance benefits)
   • We can’t reform or improve a market that doesn’t exist

2. Create more certainty, flexibility, and choice for consumers
   • Allow subsidies off exchange to help people with no or limited plan choices
   • Improve risk pools
   • Health care transparency
   • Address cause of rising premiums and deductibles – rising health costs
THANK YOU

Joel White
President
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202-744-1806
Expanded Access Navigator

June S. Wasser, Executive Director
July 19, 2017
Rare Disease Legislative Advocates Webinar
Foundation Origins and Mission

• Created by Congress in 2007 as an independent 501(c)(3) not-for-profit organization
• Established to support and promote regulatory science to advance the FDA’s mission
• Serves as a neutral third party to coordinate public-private partnerships with and between FDA and other stakeholders
Single Patient Focus (Oncology)

- PreApproval Access
- Expanded Access
- Named Patient
- Compassionate Use
- Single Patient
- Investigational Drugs

DONT BE CONFUSED
EA Landscape is Complex and Evolving

- Increased interest/visibility—access to information about unapproved experimental new drugs still in development
  - Social media
  - Advocacy
  - Press coverage
  - FDA activities
  - Industry activities
  - Legislative activities

- Desire by patients, families, and their physicians to access those drugs despite knowledge of safety or effectiveness

- Must balance drug sponsor’s ability to provide the drug and the development needs for the entire patient community

- Almost no data on administration and utilization of EA
Why the 21st Century Cures Act?

How Many Companies Posted EA information?

- Avelere Health looked at 100 company websites by market cap size and compared public posting before and after Cures
Corporate Directory

- A directory does not exist elsewhere
- Allows the website to be a centralized resource for physicians and patients
- Will complement and link to (or extract) information collected on clinicaltrials.gov
- Provides a learning resource for companies on legislation and best practices for EA policy development
- To be useful the directory must be robust (with a high industry participation rate)
- A secure database with a webform for corporate data collection, that will be reviewed internally and published by the Foundation on the Navigator site
Next Steps

• Navigator was launched in June 2017
• Evaluate the usefulness of the program on an ongoing basis with content and directory refresh/updates
• Consider expanding the Navigator resources beyond oncology (e.g., rare diseases, etc.)
• Monitor continuing legislative and other efforts related to single patient EA
• Additional fundraising for program expansion and sustainability
• Encourage companies to submit data to the directory
• Collect user feedback
Requesting Physician and Patient Feedback

Was this helpful?

Yes
No

Additional Feedback

Please provide additional feedback

Submit
Thank you
PDUFA VI Update

Ryan Hohman
Vice President – Public Affairs
Friends of Cancer Research
Prescription Drug User Fee Authorization

• First signed into law in 1992, the Prescription Drug User Fees Act, or PDUFA, was passed to address growing concern among drug developers, regulators, and patients that the FDA review and approval process was taking too long due to insufficient funding.

• The law supplements FDA funding by requiring developers to pay user fees when they submit drugs for review. In return, the FDA sets target completion times for drug review (ten months for standard review or six months for priority review), ensuring that most New Drug Applications are addressed in a timely manner.

• The bill now addresses user fees for drugs, generics, biosimilars, and medical devices

• In 1992, the FDA took 27 months on average to review a new drug. By 2012, that number had plunged to 13.3 months. In 2011, user fees paid for an estimated 65% of the drug approval process.

More than 50%

of new medicines now launch in the United States, as opposed to just 8% before PDUFA began in 1992.

*The Campaign for Modern Medicines
PDUFA VI Agreement

- In 2016, the FDA, industry, and other stakeholders produced an agreement letter surrounding user fee reauthorization and highlighted important programmatic goals. This year’s agreement letter:

- **Benefits Patients**
  - Strengthens efforts to incorporate patient perspectives into the drug development and review process
  - Fosters development and approval of medicines for patients with serious and life-threatening diseases

- **Advances Medical Innovation**
  - Supports development and application of 21st Century regulatory science
  - Facilitates timely review of innovative treatments; enhances FDA’s biomarker pathway; Explores the use of real world evidence for regulatory decision-making

- **Builds a More Effective FDA**
  - Enhances predictability and efficiency of the human drug review and approval process
  - Builds on the success of the breakthrough, priority, and standard FDA review timelines while streamlining the review of combination medicines

- **Strengthens FDA’s drug safety system**
  - Enhances existing FDA tools and technology related to post-marketing safety of approved drugs, including providing additional resources dedicated to expanding the capabilities of the agency’s Sentinel system.

- **Promotes long-term stability and sustainability of FDA’s drug review programs**
  - Helps to ensure FDA can hire and retain a strong scientific and medical workforce to advance its public health mission
Consequences of No Passage

1) Lack of Funding

2) Layoffs

3) Increased Review Timelines

• Without the authority to collect user fees, which expires September 30, 2017, the FDA will be unable to meet its payroll obligations and will begin to lay off staff and start slowing or stopping drug and device review processes.

• Layoffs would affect 5,890 full-time employees working for the brand-name and generic user fee programs based on the most recently reported hiring levels.

• An additional 1,790 full-time employees would be affected in the medical device and biosimilar programs.

• Because user fees account for 43% of FDA’s total program level (as of FY2016), reauthorization of the user fee programs is crucial for the agency and multiple stakeholders.
PDUFA Reauthorization - FDARA

- This PDUFA Reauthorization is through HR 2430 – The Food and Drug Administration Reauthorization Act of 2017 (FDARA)

- Where are we now?
  - **July 12th, 2017:** PDUFA VI (FDARA) was passed by a voice vote in the House of Representatives and now awaits action by the Senate.
RDLA's June Legislative Webinar

Sara Chang, Director, Policy and Advocacy
Research!America’s Mission

making research to improve health a higher national priority

Research!America is an innovator in advocacy for research
Appropriations Asks - FY18

Our Asks:

• $2B increase for NIH
• $2.8B for FDA
• Appropriators release the targeted FY18 Innovation fund dollars for NIH ($496) and FDA ($60M) included in the 21st Cures Act
Why NIH and FDA

**NIH**
World’s largest public funder of biomedical research, investing funding appropriated by Congress to enhance life, reduce illness and disability

**FDA**
Responsible for protecting and promoting public health through the control and supervision of food and drug products
FY18 LHHS Appropriations Status


July 13, 2017: The bill was marked up in the subcommittee and passed on party lines.

July 19, 2017: The full appropriations committee markup is today.

Senate: TBD
FY18 NIH Appropriations

- Provides $35.2 billion in program-level funding

Increase of $1.1 billion (~3%) over FY17 omnibus funding levels

- Increase is inclusive of $496 million in funding from 21st Century Cures
- Effective (inflation adjusted) funding level is comparable to FY2010 levels
NIH Appropriations

Budget authority ($millions)

- $31,238
- $30,916
- $30,861
- $29,316
- $30,143
- $30,311
- $32,311
- $34,084

2010-2017
NIH Appropriations

Budget authority ($millions)

- 2010: $31,238
- 2011: $30,916
- 2012: $30,861
- 2013: $29,316
- 2014: $30,143
- 2015: $30,311
- 2016: $32,311
- 2017: $34,084
- 2018: $36,200

$25,000
$30,000
$35,000
$40,000

FY18 FDA Appropriations

- FDA total budget = budget authority + user fees
  - Provides $2.8 billion in budget authority,
  - and $2.4 billion from industry-paid user fees
- Increase is inclusive of $60 million in new funding from 21st Century Cures
- User fees accounted for ~46% of overall funding in FY18
  - New user fee agreements in progress now
FDA Appropriations

Budget authority ($millions)

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<td>2,720</td>
</tr>
<tr>
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<td>2,759</td>
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FDA Appropriations
Thank You!

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Connect with us
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Orphan Products Now, Accelerating Cures and Treatments (OPEN ACT)

- Has the potential to double the number of therapies approved by the FDA to treat rare diseases
- Mechanism: Repurposing therapies approved for common disease for rare indications
  - Repurposing is more cost efficient and takes less time than traditional drug development but is currently underleveraged due to lack of incentives
- Proposed policy change: provide a six month extension of exclusivity for medicine that is repurposed for a rare indication
- Exclusivity is a proven incentive to spur broader industry interest in rare and pediatric diseases
• Bipartisan legislation supported by 225 patient organizations
• Included in 21st Century Cures Act passed by the House in 2015 but not in final version of Cures which was signed into law
• Reintroduced in the House as HR 1223 in February by Representatives Gus Bilirakis (R-FL), G. K. Butterfield (D-NC) and Michael McCaul (R-TX)
  • 19 additional co-sponsors added thanks to rare disease advocates
• Reintroduced in the Senate as S 1509 by Senators Orrin Hatch (R-UT) and Robert Menendez (D-NJ)
Take Action to Advance OPEN ACT

• If your organization is not already a supporter, please sign-on.
• Contact YOUR legislators through the action alert. You only need to enter your address once to contact both Senators and your Representative.
• Share the action alert with your network in emails, newsletters and social media.
• You can find information on OPEN ACT including the action alert and list of 225 supporting organizations at everylifefoundation.org/OPEN-ACT.
From August 7th through September 1st, rare disease advocates from across the country will meet with Members of Congress in their local offices to advocate for legislation benefiting the rare disease community.

Registration for In-District Lobby Days is FREE and was extended through July 20th.
Goals of In-District Lobby Days

- Strengthen your relationships with Members of Congress and staff
- Advance legislation that would benefit rare disease patients and families, and express concern with legislation that could be harmful.

A best-practice of effective advocacy is to make your friends before you need them!

We NEED to gain allies in both parties who will champion rare disease causes and keep our community in mind when working on broader health issues.
How In-District Lobby Days Work

- You specify the dates that you are not available within the timeframe (July 31st–September 1st).
- You also specify how far you are able to travel for meetings with your Representative and Senators.
- We retained Advocacy Associates to schedule the meetings and coordinate with each advocate.
- They will do their best to accommodate as many constituents as possible when scheduling each meeting.
Prefering for In-District Lobby Days

Everyone who registers for In-District Lobby Days will be invited to a webinar next Friday, July 28th, at 2pm ET/11am PT.

• The webinar will provide detailed information including tips on how to make your meetings successful and what to research prior to your meetings.
• Key legislative issues will also be covered.
• Questions will be welcome so don’t be shy!
• If you can’t join us, you’ll be able to access the archived webinar within a day at http://rareadvocates.org/in-district-lobby-days/.
Know someone who has been a “Voice” for the rare disease community and should be recognized for their work? Visit RareVoiceAwards.org and submit your nomination!

We encourage the nomination of individuals and organizations who have gone above and beyond to become rare disease community leaders and political advocates. Nominations are considered in the following categories:

- Federal Advocacy: Agency Staff
- Federal Advocacy: Congressional Staff
- Federal Advocacy: Patient/Organization
- State Advocacy: State Legislator
- State Advocacy: Patient/Organization
- Teen Advocacy

Deadline to submit nominations is July 31st. Abbey recipients will be announced live at the RareVoice Awards. www.RareAdvocates.org
Sixth Annual RareVoice Awards

Join us to celebrate and thank Members of Congress, Congressional staff, government agency leaders and patient advocates who are policy leaders and legislative advocates for the rare disease community.

❖ Wednesday, November 15, 2017
❖ Arena Stage, 1101 6th Street, SW, Washington, DC

- 6:00 p.m. Cocktail Reception
- 6:30 p.m. Congressional Toast
- 8:00 p.m. Awards Ceremony
- 9:00 p.m. After Party Celebration
Sponsorship Opportunities Available

Thank you to our 2017 RareVoice Sponsors to date:

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PhRMA

Silver

Biomarin

Vertex

Sanofi Genzyme

Recordati Rare Diseases

Gilead

Horizon

Please contact Carol Kennedy at ckennedy@everylifetfoundation.org for levels and benefits