

NORD

National Organization for Rare Disorders

VHL Alliance Annual Family Meeting
Saturday, October 18, 2014
Rockville Hilton
Rockville, MD

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Outline...

- Background on NORD
- Overview of Affordable Care Act
- Rare Action Network[®]
- Advocacy & 21st Century Cures



Introduction to NORD...

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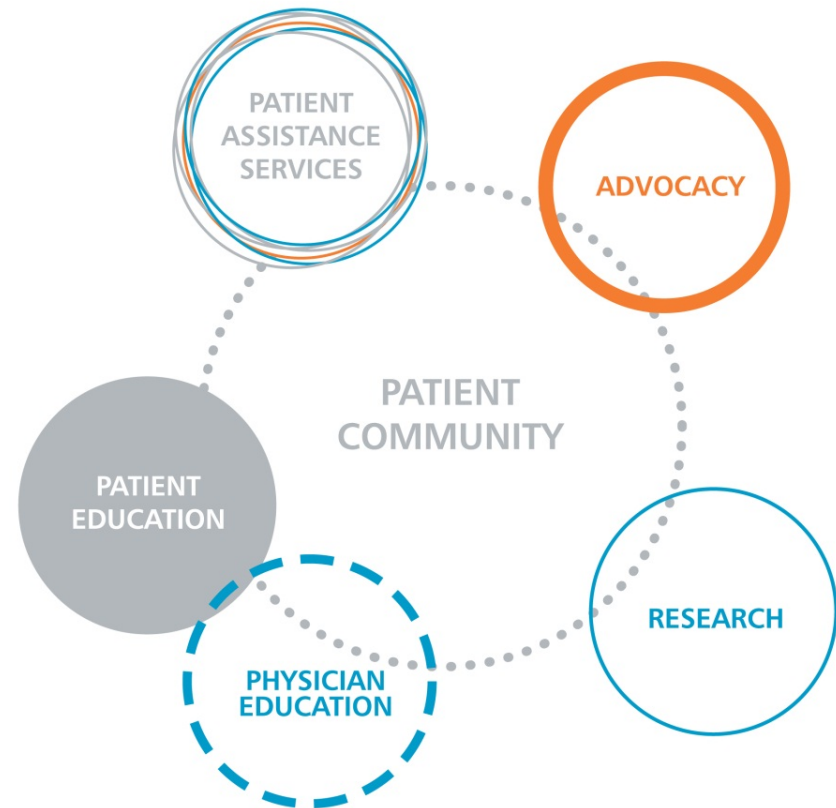
NORD's Guiding Principles...

- Raise awareness and recognition of the challenges faced by people living with rare diseases and the costs to society;
- Support a nation where people with rare diseases can secure access to diagnostics and therapies that extend and improve their lives;
- Foster a social, political, and financial culture of innovation that supports the basic and translational research necessary to create diagnostic tests and effective therapies for all rare disorders;
- Create a regulatory environment that encourages development and timely approval of safe and effective diagnostics and treatments for individuals with rare diseases



What NORD Does...

- Advocate
- Educate
- Connect
- Support Innovation
- Promote Access
- Provide Assistance



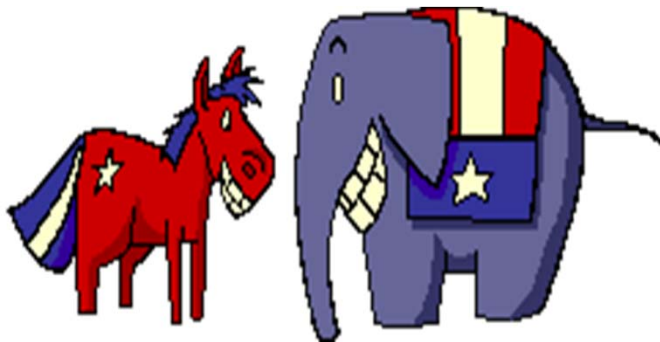
Major Accomplishments...

- | | | | |
|--------|--|------|---|
| 1984/5 | NORD supports amendments to Orphan Drug Act to improve practical applicability | 2006 | First meeting of the International Council on Rare Diseases and Orphan Products (ICORD) in Stockholm |
| 1987 | NORD establishes first-ever patient assistance program | 2007 | FDA and EMEA adopt common application form for sponsors seeking orphan drug designation of medicines |
| 1989 | National Commission on Orphan Diseases issues report on experiences of patients, families affected by rare diseases | 2008 | Social Security Commissioner announces " Compassionate Allowances Program " to fast-track assistance for patients with rare and severely disabling diseases. |
| 2000 | ClinicalTrials.gov established by NIH (partly in response to NORD and others in the patient community) | 2011 | NORD documents FDA's flexibility in review of non-oncologic orphan drugs from 1983 to 2010. |
| 2002 | Rare Disease Act signed into law (codifies NIH Office of Rare Diseases Research; lays groundwork for the NIH Rare Diseases Clinical Research Network) | 2012 | NORD's advocacy efforts facilitate passage of the FDA Safety and Innovation Act |



Public Policy...

- U.S. Congress
- Food & Drug Administration
- National Institutes of Health
- Center for Medicare & Medicaid Services
- Social Security Administration
- Patient Advocates
- Industry



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Affordable Care Act...

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Ever Play Whack-A-Mole?...



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What's in the ACA?...

- Creates the Health Insurance Marketplace
- Requires insurance to cover people with pre-existing health conditions
- Holds insurance companies accountable for rate increases
- Makes it illegal for health insurance companies to arbitrarily cancel your health insurance
- Protects your choice of doctors – sometimes!
- Covers young adults under 26
- Provides free preventive care
- Ends lifetime and yearly dollar limits on coverage of essential health benefits



Essential Health Benefits...

Essential health benefits are minimum requirements for all plans in the Marketplace. Plans may offer additional coverage

- Ambulatory patient services
- Emergency services
- Hospitalization
- Maternity and newborn care
- Mental health and substance use disorder services, including behavioral health treatment
- Prescription drugs
- Rehabilitative and habilitative services and laboratory services
- Preventive and wellness services and chronic disease management
- Pediatric services



Free Preventative Services...

All Marketplace plans and many other plans must cover the following list of preventive services without charging a copayment or coinsurance. This is true even if you haven't met your yearly deductible. This applies only when these services are delivered by a network provider

- **Abdominal Aortic Aneurysm one-time screening** for men of specified ages who have never smoked
- **Alcohol Misuse screening and counseling**
- **Aspirin use** to prevent cardiovascular disease for men and women of certain ages
- **Blood Pressure screening** for all adults
- **Cholesterol screening** for adults of certain ages or at higher risk
- **Colorectal Cancer screening** for adults over 50
- **Depression screening** for adults
- **Diabetes (Type 2) screening** for adults with high blood pressure
- **Diet counseling** for adults at higher risk for chronic disease
- **HIV screening** for everyone ages 15 to 65, and other ages at increased risk
- **Immunization vaccines** for adults--doses, recommended ages, and recommended populations vary
- **Obesity screening and counseling** for all adults
- **Sexually Transmitted Infection (STI) prevention counseling** for adults at higher risk
- **Syphilis screening** for all adults at higher risk
- **Tobacco Use screening** for all adults and cessation interventions for tobacco users



Access to Specialists – Maybe!...

- *Depending on the plan you choose* in the Marketplace, you may be able to keep your current doctor
- Different plans have different networks and providers
- Most health insurance plans offered in the Marketplace have networks of hospitals, doctors, specialists, pharmacies, and other health care providers. Networks include health care providers that the plan contracts with to take care of the plan's members.
- Depending on the type of policy you buy, care may be covered only when you get it from a network provider
- If staying with your current doctors is important to you, check to see if they are included before choosing a plan



Enrolling...

- Open enrollment starts November 15
- Apply for Medicaid and CHIP any time
- Special enrollment period
 - Moving to a new state
 - Changes in income
 - Changes in family size – marriage, divorce, new baby



Where Do I Go for More Information?...

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The Sky is Falling!



*It pays
to be
prepared*





<http://www.rarediseases.org/patients-and-families/state-by-state-insurance-information>

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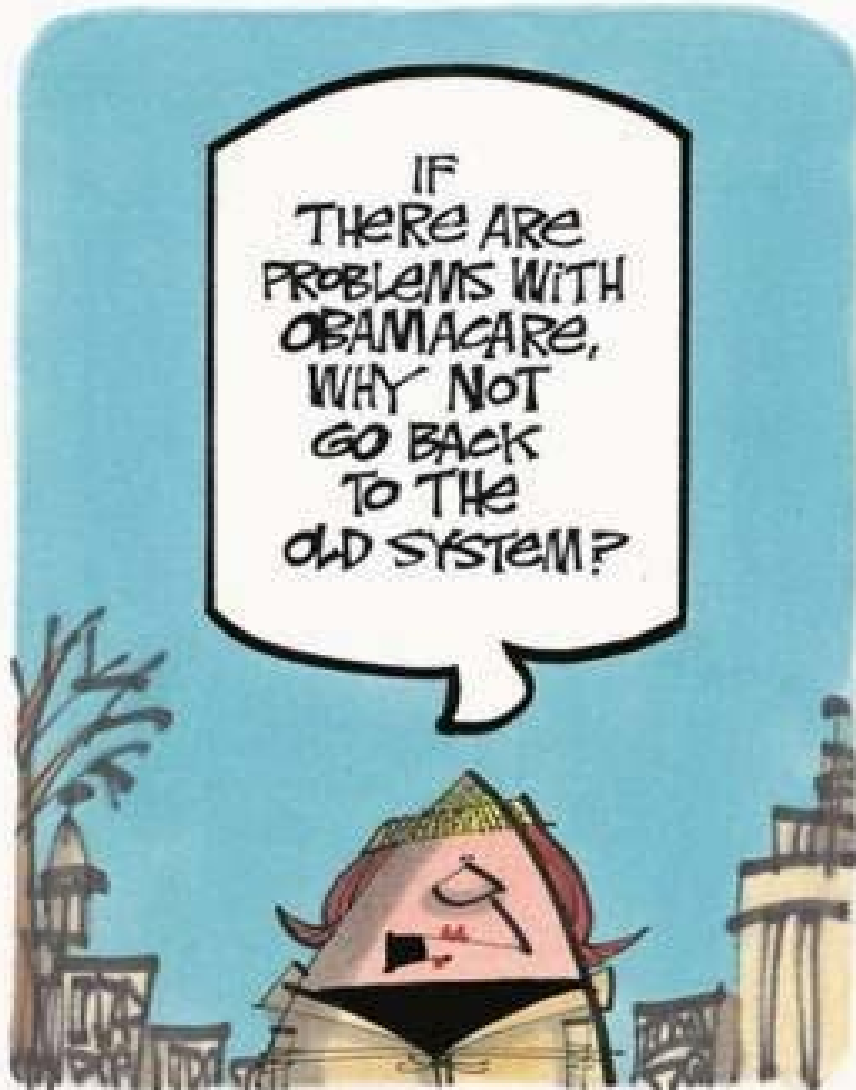


Kaiser Family Foundation...

- <http://kff.org/health-reform/faq/health-reform-frequently-asked-questions/>
- What is the health insurance Marketplace?
- How do I find my state Marketplace?
- Who can buy coverage in the Marketplace?
- I live in one state, but drive across the border every day to work in a different state. What Marketplace should I use to buy coverage
- I'm eligible for health benefits at work but want to see if I can get a better deal in the Marketplace. Can I do that?



ASK A CARTOONIST:



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judger@ksu.edu Judge

A.



THE OLD SYSTEM.

Rare Action Network [®]

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What is RAN[®] ?...

NORD initiative to mobilize patients around the country to advocate for policies that support the needs of patients with rare diseases

It will predominantly work at the state level, where more and more decisions are being made concerning health care, especially therapies and services for patients with rare diseases

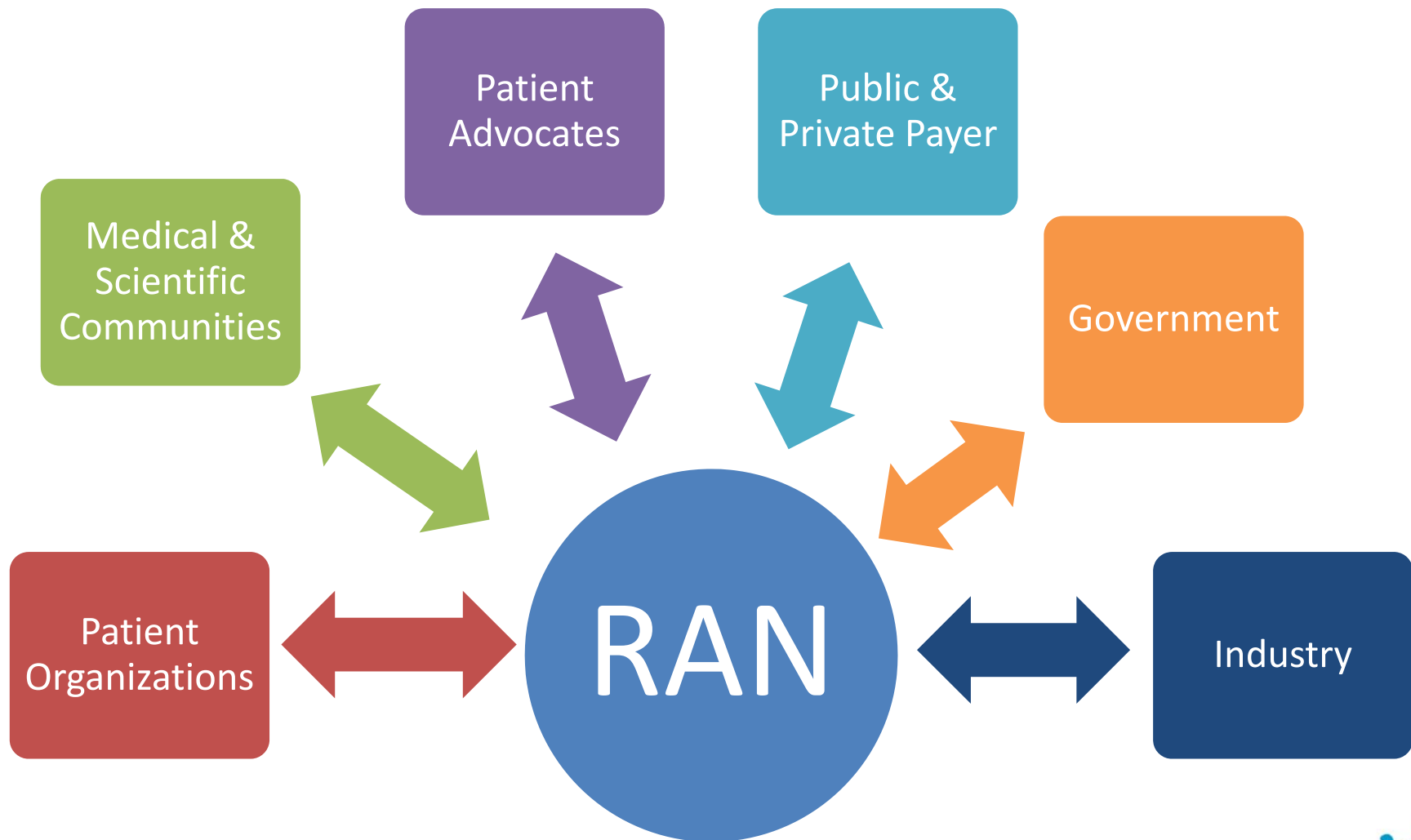


Goals...

- Increase awareness of the challenges faced by the rare disease community
- Build a unified network of rare disease advocates to take action on issues that impact patients with rare diseases
- Address issues of access and coverage to essential treatments and therapies for patients with rare diseases



Rare Action Network[®] Partners...



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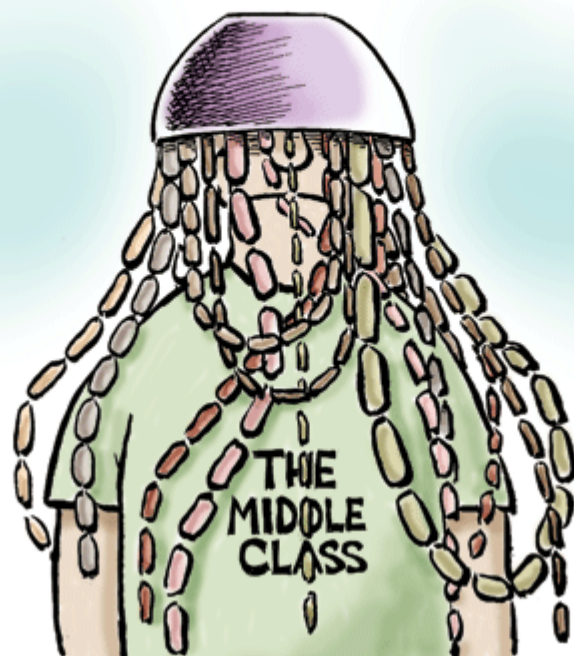


Advocacy Changes Laws & Public Policies...

- Advocacy is a democratic tradition
- Policymakers need your expertise
- Lobbying advances your cause
- Advocacy builds public trust



THE LEGISLATIVE SAUSAGE-MAKING PROCESS



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Out of 7,207 bills and joint resolutions introduced during this Congress, less than 200 have become law - fewer than in any other Congress since at least the 1970s

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“Deadlock in Congress Appears to Worsen as Midterms Loom”...



The U.S. House of Representatives has voted at least 54 times to repeal the Affordable Care Act

New York Times
July 1, 2014
By Carl Hulse



Next Steps...

- Increase your understanding of state and federal laws & regulations
- Get to know your state & federal legislators
- Build partnerships & collaborations
- Cure the “**What’s In It For Me**” syndrome



U.S. House of Representatives 21st Century Cures...

- Comprehensive look at steps to accelerate the pace of cures in the U.S.
- **DISCOVER** clues in basic science
- Streaming the drug & device
DEVELOPMENT
- Unleashing the power of digital medicine & social media at the **DELIVERY**
- <http://energycommerce.house.gov/cures>



Thank You!!

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