PATIENT'S PERSPECTIVE

CLINICAL TRIALS

RESEARCH ADVOCACY AND

Expanded Access Programs

New Models for Stakeholder Collaboration, Program Design, Supply Equity and Access for Investigation Drugs

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WHO HAS RIDDEN ON A ROLLER COASTER?
MY EXPERIENCES: HERE'S THE SET UP

• You have blood cancer!
• Rare, not yet curable
• Waldenstrom’s Macroglobulinemia

• No radiation, no surgery
• No known chemotherapy
• Indolent – 5 Year Outlook

What would you do?
• Become an e-Patient
E-Patients

- Electronically connected
- Equipped with the skills to help manage their own condition
- Enabled to make choices about self-care
- Empowered to ask relevant questions, not passive
- Engaged in their own care
- Equals with physicians involved
- Emancipated
- Expert about their disease, some: Patient Opinion Leader
- Experienced, there's no substitute
- Educated, credible (most important)
Disclosure statement of financial interest

• I have no actual or potential conflict of interest in relation to this presentation.

• I will not be discussing
  – “off-label” uses of any medications

• I am not a Medical, Scientific or Life Sciences Pro, this is only my personal patient perspective.
GOALS FOR THIS PRESENTATION

• Give you my personal e-Patient perspective
  – Real world observations, why so optimistic?

• Urgent need/solution - Clinical Trials, and EAPs
  – May be safer-more effective option for treatment

• Some comments on Personalized Medicine
  – Good news for Patients & Caregivers

• Some info that could save your life!
CAUTION: E-PATIENTS/ADVOCATES

- Great American Philosopher
- “Advocates are like a box of chocolates, ya never know....”
- Some are vocal, active in HCSM
- May crash your event, mad
- Misinformed, cyberchrondriacs
- Some focus on what’s wrong
- Man on the street, except for their experiences!
HOW TO BECOME AN E-PATIENT ADVOCATE

Attend Conferences

Speak at Conferences

Present Posters, defend your message

From Adversity to Advocacy
E-PATIENTS WANT TO BECOME OLD PEOPLE

Introducing "Bob" and Jack
CAN YOU IDENTIFY WITH THIS?
“Laughter is the best medicine”
- **Personal:** Married 45 years to my wife and caregiver Jan! ❤️
- Three **fabulous married daughters**, Laurie, Patti and Karen
- Three outstanding sons-in-law, perfect twin **granddaughters**, one more, more twins on way!
- **United States Navy** Veteran, **University of Rhode Island**, MIT antique radio & phonographs
- Consultant/POL to: Venture Investors, biopharma, CRO, Conference producers

- **Patient Advocacy:**
  - IWMF **Support Group Leader** New England, fundraising for research
  - Co-Chair Dana Farber Cancer Institute (DFCI) **Patient Family Adult Council** (PFAC)
  - Member Dana Farber Cancer Institute Jimmy Fund- spokesman, TV/radio commercials
  - Leukemia & Lymphoma Society **Support Group Speaker, Team in Training** recruit speaker

- **Professional Advocacy:**
  - Member American Association for Cancer Research: **AACR Scientist↔Survivor Program**
  - Member American Society of Clinical Oncology: **ASCO Research Advocate Scholar**
  - Member Speaker Education Network to Advance **Clinical Trials ENACCT, ESCAPE Graduate**
  - Drug Information Association **DIA Advocate**, NORD/National Organization for Rare Disorders

- **Legislative Advocate:** Oral Parity Chemo, Step Therapy, Biosimilars, educate lawmakers!
- **Rare Disease Legislative Advocate (RDLA)**, effective Congressional arm-twisting, face to face
- **AACR Rally for Medical Research 2013/4 Capitol Hill Meetings, LLS State and Federal**
- Gracefully retiring **Research Analyst** becoming a **Research Advocate**, It’s time to give back.
LIVING WITH A NOT YET CURABLE BLOOD CANCER IS LIKE A ROLLER COASTER RIDE

• Mostly good days some not-so-good days

• Emotional Highs & Lows

• Uncertainty, clarity

• So I became an e-Patient
Roller Coaster ride

- Plasmapheresis
- Rituximab
- LY-31765
- Carfilzomib CaRD
- Velcade
- RAD001
- LBH589
- Ibrutinib?
- Cyclophosphamide
BLOOD CHEMISTRY  01/09 – 01/15
SITUATION, 2007 BC*

• Considered myself
  – Strong, Healthy
  – Exercise, Diet
  – Cholesterol LDL 120, HDL 60
  – Blood Pressure 120/80
  – Good “biomarkers”
  – Good immune system
  – Rarely sick
  – Gracefully Becoming Bob

• The only medication
  – 81 mg day aspirin regimen

* Before Cancer
Situation, 2007 AD*

- Serum IgM concentration of 6000 milligrams per deciliter, normal is 55 to 375 mg/dl
- Any symptoms?
  - Tired walk to/from work
  - Nose bleeds after a walk
  - Fatigue, “bone tired”
  - More ‘naps’, sleeping longer
  - What’s a hot flash?
  - Headaches
  - Migraine aura (flashing lights)
  - Couldn’t concentrate, study

* After Diagnosis
TREATMENT OPTIONS

• Conventional Chemotherapy
  • alkylation agents (Chlorambucil, Bendamustine)
  • nucleoside analogs (Cladribine, Fludarabine)
  • Long term effect on bone marrow, collect stem cells?

• Biologic, targeted Chemotherapy
  • Monoclonal anti-body, Proteasome Inhibitors, mTOR Inhibitor, HDAC Inhibitor, Pi3K Pathway Inhibitor
  • BTK Inhibitor (TKI) maybe soon

• Combination of the above

• e-Patients explore a variety of options
**WHY I'M INTERESTED IN EXPANDED ACCESS**

- Mine is a repeating relapsed refractory cancer
- “Not yet curable”, indolent, active, symptomatic
- Breakthrough designation from FDA, horizon
- Awaiting approvals, trials, expanded access
- Until last week, no FDA approved drug
  - Imbruvica, Bruton’s Tyrosine Kinase Inhibitor
  - MYD88 somatic mutation L265P, not for CXCR4
  - “Financial Toxicity”, co-ins., not on formularies
A quick peek inside a B cell

Image courtesy Genentech
How far have we come in healthcare?
**How far have we come in healthcare?**

- **Great Horse Manure Crisis 1894**
  - Predict shutting down London and New York
  - From horse power to *horsepower* (automobile)
  - Worried more about the effects of the system
    - “what’s on the street” rather than “who’s in the ambulance”

- **Completely change our way of thinking**
  - Focus on target inside: the patient, not the system
  - Get away from empirical therapy, move to genomics
**What is Personalized Medicine?**

- Also *Precision, Individualized*
- Use of genetic and genomic information
- Ultimate promise of the Human Genome Project
  - Improved our understanding of genes, how they vary
- Not just disease, *your* disease.
- Molecular level, pinpoint what drives the growth of cancer cells, *what are your* abnormalities
- Predictive abilities, help physician choose most effective clinical trial or treatment option.
Empiric and Genomics-based Therapy

Empiric Therapy
1 2 3 4 5

Drug A

If Drug A Doesn’t Work

Drug B

Genomics-based Therapy
1 2 3 4 5

Genetic Testing
1 2 3 4 5

Drug A

Drug B

Drug C

Graphic concept: Courtesy of Research Advocacy Network
EXAMPLE OF GENETIC TESTING

Take a blood sample

The sample contains cells

The cells contain DNA

The test looks for misspelling in DNA

Image: Courtesy of Research Advocacy Network
SCREENING, MOST LIKELY TO RESPOND?

Diagnostic Test?

Diagnostic Positive +
Selected for targeted therapy

Diagnostic Negative -
Not selected for targeted therapy

Screening patients with the appropriate diagnostic tests
PERSONALIZED MEDICINE

• Use info about genes, proteins & our environment to prevent, diagnose and treat disease¹.

• Biomarker testing:
  – patient characteristics indicate normal/abnormal processes & treatment response
  – May help identify who are more likely to respond
  – WM Patients, ~90% show high prevalence of the MYD88 L265P somatic mutation²


We’re not there yet!
COMPANION DIAGNOSTICS¹

- Companion Diagnostics (CDx) issues
  - Central to the development of Personalized Meds
  - Tests to identify who are likely to respond to drug
  - Help enhance the use of targeted therapies
    - Who funds this research?
    - Will Gov’t & insurance providers pay for these tests?
    - Diagnostic companies and Drug companies have very different business models.
    - Regulatory issues emerging, will industry educate?

¹ From Tufts University Center for the Study of Drug Development (CSDD Report 4/2013)
CALL TO ACTION

• More education about Clinical Trials (safe, effective)
  – Genetics and genomics is the game changer
• Payers need to see “value” and cost savings of:
  – Molecular level biomarker companion diagnostics CDx
  – Get Laboratory Developed Tests, ‘standards, regulated’
• Regulators and lawmakers, co-operate
  – Need our help, education, engagement, don’t blame/bash!
  – Continue and expand “fast track” programs, no need to wait
  – Important legislation: Oral Parity Chemo, Step Therapy, Specialty Tiers, Biosimilars clarification, Seniors face CMS issues, “donut hole”, CMS formulary issues, VA formulary
• Partner with patients “participatory data”, give data back
CALL TO ACTION

• Why support Pharma, Biotech, Life Sciences industry?
  – We can dramatically reduce the overall cost of healthcare.
  – Personalized “precision” medicine is a game changer.
  – We can’t sustain the way we do remedial care,
    • Solution is preventive and prognostic care.
  – Best care for the citizens of USA, a tech leader!

• Support and enable this industry
  – Creates jobs, feeds our families, pays our taxes
  – Biosimilair legislation in particular needs clarification
    • Preserve Doctor/patient relationship  • It’s not like generics, not the same
HOW MUCH TIME DO I HAVE?
CLINICAL TRIALS
Myths & Misconceptions

From Ken Getz, Tufts University, CenterWatch
**Why I participate in Clinical Trials**

- Working on my situation
- Not for any altruistic purpose
- Ongoing rational, contemplated decisions
- Goal: survive long term, forward-looking odds
- Smart enough to weigh the risks/rewards
- Strong enough to take some chances
- Confident in my Team
- Closely monitored
- Best hope for a solution
IMPACT HEAVILY REGULATED ENVIRONMENT

• Distribution channel doesn’t facilitate open communications: Biopharma ↔ Patient

• Regulatory environment prevents open communications

• Life Sciences, Biopharma industry is risk averse

• HIPAA & privacy compliance encourages silence!
**Impact Heavily Regulated Environment**

- Regulatory financial cost, compliance

- Regulations fail to keep up with state-of-the-art

- Regulatory tax base, US Fed’l, State 40% of profit

- ACA “Obamacare” Regs. impact not felt yet, but
  - 2.3% excise tax medical devices = + 15% tax on profit
  - Overall U.S. corp. tax rate will exceed 50%, most states
  - Seniors and Veterans w/ chronic or rare illness hit hard
**Impact Heavily Regulated Environment**

- “Financial toxicity” for patients
- Pharmaceutical/biologics cost vs Medical costs
  - Oral chemotherapy parity legislation, commercial vs gov’t
  - Step Therapy, “fail first”
  - Specialty tiers, 1, 2, 3 fixed $ amount, co-payment
  - Tier 4, co-insurance 20-40% of drug cost
    - Ibrutinib (TKI) Targeted, Pill-form $10K, $2813 Co-Ins.
    - Carfilzomib 2X Infusion $25K infusions, $0 Co-Ins.
- Jack’s Personal situation
  - Drugs not FDA Approved, not on CMS or VA Formulary
  - Clinical Trials or EAP
Facilitate direct engagement, patient

- Patient data, close the loop
  - Send a “Thank you” card, acknowledge when you can.
  - Start with biomarkers, “how am I doing?”
  - Physician ↔ Patient engage

- Partner with patients
  - “participatory data”, give data back
  - In research, epidemiology, pharma, payers, providers

- EMR/EHR Progress
  - Patient Gateway, Epidemiology study, Billing, Services
  - Care Plan, Care Team – who did you see? History
Facilitate direct engagement, patient

- Adherence & engagement thru more personalized service
  - “Patient Centered” in care/clinic and research/lab
- Emerging eCOA/ePRO Systems
  - electronic Clinical Outcome Assessments (eCOA)
  - electronic Patients Reported Outcome (ePRO)
- Simplify and automate biomarker/data collection
- Ease of use, “fun” to engage patients, mobile devices
  - Auto-Diary, logs, schedule, care plan
  - Answer key question: How am I doing? This engages.
- Dashboard, Questionnaire, Prompts
THANK YOU!

Expanded Access Programs

Never Ever Give Up!

E-mail me:
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Never Ever Give Up!