DON'T FORGET ABOUT THE PATIENT!
LISTENING TO PATIENTS AND INVOLVING THEM IN RESEARCH

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Chris Kula-Przezwanski
Derek Stewart
Dean Summerfield
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clinical | commercial | consulting | capital

Why we must not forget the patient!

On behalf of Derek Stewart

clinical | commercial | consulting | capital
We are all patients, carers and people!

- **Purpose**
- **Customer**
- **Reminder**
- **People**

Our shared interests

- **Relevance**
- **Appropriateness**
- **Practicality**

Improving research

In the UK we have...

- Public members of major funding board and committees assessing applications
- Public representation on Clinical Studies Groups
- Patients/carers identifying unanswered research questions.
- Service users working with researchers to design and deliver research
- Service users as co-applicants on research proposals
- Patients, carers and public involvement in commissioning services that are based on quality evidenced based research.
For now and the future

There is a need for...

• Greater openness and transparency
• Open access to data
• More honesty in publication and reporting
• Better behaviour
• A new dialogue – between industry and the public

How do we involve patients more?

Do you agree that the patient voice should be more prominent in development, approval and access?
Involving patients – the stakeholder perspective

Dean Summerfield

What do payers want?

>Payers’ emphasis is on patient-oriented rather than product-oriented evidence

<table>
<thead>
<tr>
<th>Feature</th>
<th>Percentage Agreeing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Improved quality of life of patient</td>
<td>52%</td>
</tr>
<tr>
<td>Whether it addresses an unmet medical need</td>
<td>39%</td>
</tr>
<tr>
<td>Improved longevity of patient</td>
<td>36%</td>
</tr>
<tr>
<td>Costs compared to competing products</td>
<td>29%</td>
</tr>
<tr>
<td>Degree of informed efficacy over existing products</td>
<td>26%</td>
</tr>
<tr>
<td>Total patient outcomes</td>
<td>26%</td>
</tr>
<tr>
<td>Potential number of patients who could use the drug</td>
<td>10%</td>
</tr>
</tbody>
</table>

>"When we talk about value, we are talking about...better outcomes for patients [and] better health of populations"

- Dr Ling, CMS

In the same study, regulators shared very similar perspectives to payers

Source: Economist Intelligence Unit: “The Value Challenge”, February 2012
Most pharma companies support the shift...

In a recent survey, over half of pharma companies supported shifting from a medically defined assessment of treatments to a more patient-orientated approach.

![Support](support.png)

**Support**: 55%

**Neither support or oppose**: 30%

**Oppose**: 15%

Source: Quintiles New Health 2012 Report, “Rethinking the Risk Equation in Biopharmaceutical Medicine

... emphasising patient access and targeting

There is a consensus about the benefits and improvements within the field that could come from this potential change.

<table>
<thead>
<tr>
<th>Benefit</th>
<th>Percentage agreeing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Enhanced patient population targeting</td>
<td>70%</td>
</tr>
<tr>
<td>Increased patient access</td>
<td>65%</td>
</tr>
<tr>
<td>Drugs available more quickly</td>
<td>63%</td>
</tr>
<tr>
<td>Improved outcomes data</td>
<td>60%</td>
</tr>
<tr>
<td>Increased sales for manufacturers</td>
<td>53%</td>
</tr>
<tr>
<td>Shorter timeline to market for manufacturer</td>
<td>52%</td>
</tr>
<tr>
<td>Improved adherence</td>
<td>47%</td>
</tr>
</tbody>
</table>

“Considerable evidence suggests that patient engagement can improve their experience and satisfaction and also can be effective clinically and economically.”

Sources: Quintiles New Health 2012 Report, “Rethinking the Risk Equation in Biopharmaceutical Medicine

But concerns persist, particularly around costs and assessing real-world outcomes.

What are the biggest issues involved with a more patient oriented approach?

<table>
<thead>
<tr>
<th>Issue</th>
<th>Percentage Agreeing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Administration costs too high</td>
<td>71%</td>
</tr>
<tr>
<td>Difficult to pre-assess outcomes in real-world rather than clinical trials</td>
<td>69%</td>
</tr>
<tr>
<td>Difficult to agree definition of success with payers</td>
<td>68%</td>
</tr>
<tr>
<td>Regulatory risk</td>
<td>68%</td>
</tr>
<tr>
<td>Information produced might be useful to competitors</td>
<td>63%</td>
</tr>
<tr>
<td>Difficult to accurately measure success in a performance based risk sharing agreement</td>
<td>60%</td>
</tr>
<tr>
<td>Have to shift post-marketing resources from enhancing to proving the product</td>
<td>54%</td>
</tr>
</tbody>
</table>

Source: Quintiles New Health 2012 Report, "Rethinking the Risk Equation in Biopharmaceutical Medicine"

Health care professionals (HCPs) on the other hand may not be as convinced.

"Patients will want inappropriate or expensive treatments if you give them a choice"

"There's no time to do it"

"We are struggling to engage patients to attend our focus groups"

"Guidelines can help inform, but they cannot replace context-responsive professional judgement"

"We already do it"

"Our patients don't want it"

**What’s the patient perspective?**

**What is the most important value of prescription medication?**

- Improving people’s QOL: 51% US, 60% UK
- Helping people live longer: 14% US, 24% UK
- Safety: 5% US, 8% UK
- Side effects: 4% US, 4% UK
- Other*: 4% US, 27% UK

Source: Quintiles New Health 2012 Report, “Rethinking the Risk Equation in Biopharmaceutical Medicine

**Regarding access to medicines**

- We take too long to make drugs available: 87% agree, 71% agree
- Patients should be able to choose to take potentially risky medication even if it is not approved for use: 81% agree, 72% agree

**Patients and payers have the similar perspectives of value – Improving QOL and longevity of life.**

**When questioned regarding clinical trials, patients want to be more involved but are uninformed**

- Not interested at all: 16%
- A little interested: 13%
- Somewhat interested: 31%
- Very interested: 17%
- Extremely interested: 23%

- Not at all familiar: 35%
- A little familiar: 29%
- Somewhat familiar: 27%
- Very familiar: 5%
- Extremely familiar: 4%

- Not aware of clinical trial opportunities: 68%
- Concerns receiving placebo: 16%
- Concerns losing current medical coverage: 12%
- Concerns receiving an experimental drug: 11%
- Concerns study length, time, and costs: 6%
- Other: 2%

40% of patients say that they are very or extremely interested in participating in clinical trials.

Only 9% are very or extremely familiar with the concept of clinical trials.

68% of patients say they’ve never been made aware of opportunities to participate in trials.

Source: MediGuard.org UK members survey, May 2011
How do we involve patients more?

What successful approaches have you used to engage patients in the decision making and development process?

WORKING WITH PATIENTS TO ADVANCE RESEARCH

Elisa Cascade
The need for patient involvement in research

> Researchers are being asked to do more with less
  - Decrease in availability of funds for registration and non-registration research
  - Increase in stakeholder demand for real-world data post-approval

> While sometimes overlooked as a stakeholder, patients are:
  - Motivated to help others by participating in research
  - Interested in patient quality of life data in addition to safety and efficacy

> Direct patient interaction has the potential to enable a paradigm shift in the conduct of interventional and observational research

<table>
<thead>
<tr>
<th></th>
<th>Faster Enrollment</th>
<th>Increased Retention</th>
<th>Decreased Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Upfront study design more patient-friendly</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Recruitment and retention activities relevant to patients</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Patient outreach to supplement recruitment</td>
<td>✓</td>
<td></td>
<td>✓</td>
</tr>
<tr>
<td>Patient engagement during study (and beyond)</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Remote visits (hybrid virtual study designs)</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
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</tbody>
</table>

68% of UK patients don't participate in research because they are unaware of studies.

Conducting research with patients

Driving cost and time efficiencies through patient relationships

Step-wise Process for Improving Efficiencies with Digital Patients

Now

Clinical Study Support
Observational PRO Data
Observational PRO+EMR+Lab
Hybrid Virtual Clinical Trials

Next

Direct-to-Patient Involvement

Clinical Study Support
- Patient protocol assessment for feedback on:
  - I/E criteria
  - Barriers & facilitating messages
  - Supplemental patient recruitment
  - Retention
  - Alumni communities

Observational PRO
- Direct recruitment of patients (no sites)
- On-line patient consent & screening
- Capture of ePRO data

Observational PRO+EMR+Lab
- Direct outreach to patients
- Online consent and ePRO
- Patient consent for medical record access
- Patient provision of DNA or blood sample (all without sites)

Hybrid Virtual Trials
- Direct patient outreach & pre-qualification
- Physician visit for screening & randomization
- Remote visits with ePRO; in-person visits for physician endpoints or risk
- Would work well for pragmatic trials
Case study example: Wales Cholesterol PRO+EMR

**Background**
- **Objective:** Build a UK data-rich environment to measure outcomes
- **Approach:** Conducted study to demonstrate PRO+EMR link with SAIL data warehouse in Wales, UK
  - In 6 weeks, recruited 240 cholesterol patients who completed PRO assessments and consented to share identifiers
  - Identifiers provided to NHS Wales to create pseudo-identifier bridge into SAIL data warehouse
    - PRO data combined with electronic data stored in SAIL at Swansea Univ. for all Wales

**Findings**

<table>
<thead>
<tr>
<th>Patients (224 of 240)</th>
<th>Diagnosis (89 of 91)</th>
</tr>
</thead>
<tbody>
<tr>
<td>93%</td>
<td>98%</td>
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</table>

**Conclusions**
- 1/3 cholesterol is above target
- ~50% a little or not at all knowledgeable about their condition
- 60% non-adherent based on MARS-5


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Benefits and limitations of direct-to-patient research

**> Benefits**
- More rapid study launch and shortened timelines
- Decreased costs compared to the physician-centric model
- Strong patient interest in method
  - Helping others
  - Alignment of patient incentives
  - Comprehensive condition monitoring and tracking
- Patient perspective on the "why’s"

**> Limitations**
- Questions about data quality
  - Verification of patient diagnosis
  - Self-reported data (e.g., LDL)
  - Length of recall
  - Lack of randomization
- No physician involvement
- Ethics undefined in some countries
MANAGING RELATIONSHIPS DIRECTLY WITH PATIENTS

Driving Speed and Cost Savings

> Value for all Stakeholders:
  * Patients
  * Payers
  * Sponsors
  * Investigators

Future: Virtual Clinical Research

Driving greater efficiencies through direct patient engagement

> Speedy and Efficient Enrollment
  * Direct-to-patient recruitment
  * On-line screening and consent

> Continued patient engagement
  * Retention activities
  * Remote lost to follow-up tracking

> Next stage in patient involvement = virtual consent and/or remote patient visits
  * Reduces site and patient burden
  * Reduces investigator grant fees
  * Reduces site monitoring visits

Sample Hybrid Virtual Pragmatic Trial

Patient assessments

Month 1  Month 2  Month 3  Month 4  Month 5  Month 6  Month 7  Month 8  Month 9  Month 10  Month 11  Month 12

Consent

Month 1

Study News Letter

Month 2

Remote Pt. Follow-up

Month 3

Patient Visit

Month 4  Month 5  Month 6  Month 7  Month 8  Month 9  Month 10  Month 11  Month 12
How do we involve patients more?

What do you think the next major steps will be in patient-centred research?

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