



Regulator & Industry Workshop on Patient Engagement & Involvement:

Setting the scene - industry: industry processes for patient involvement, organisational setup, roadblocks

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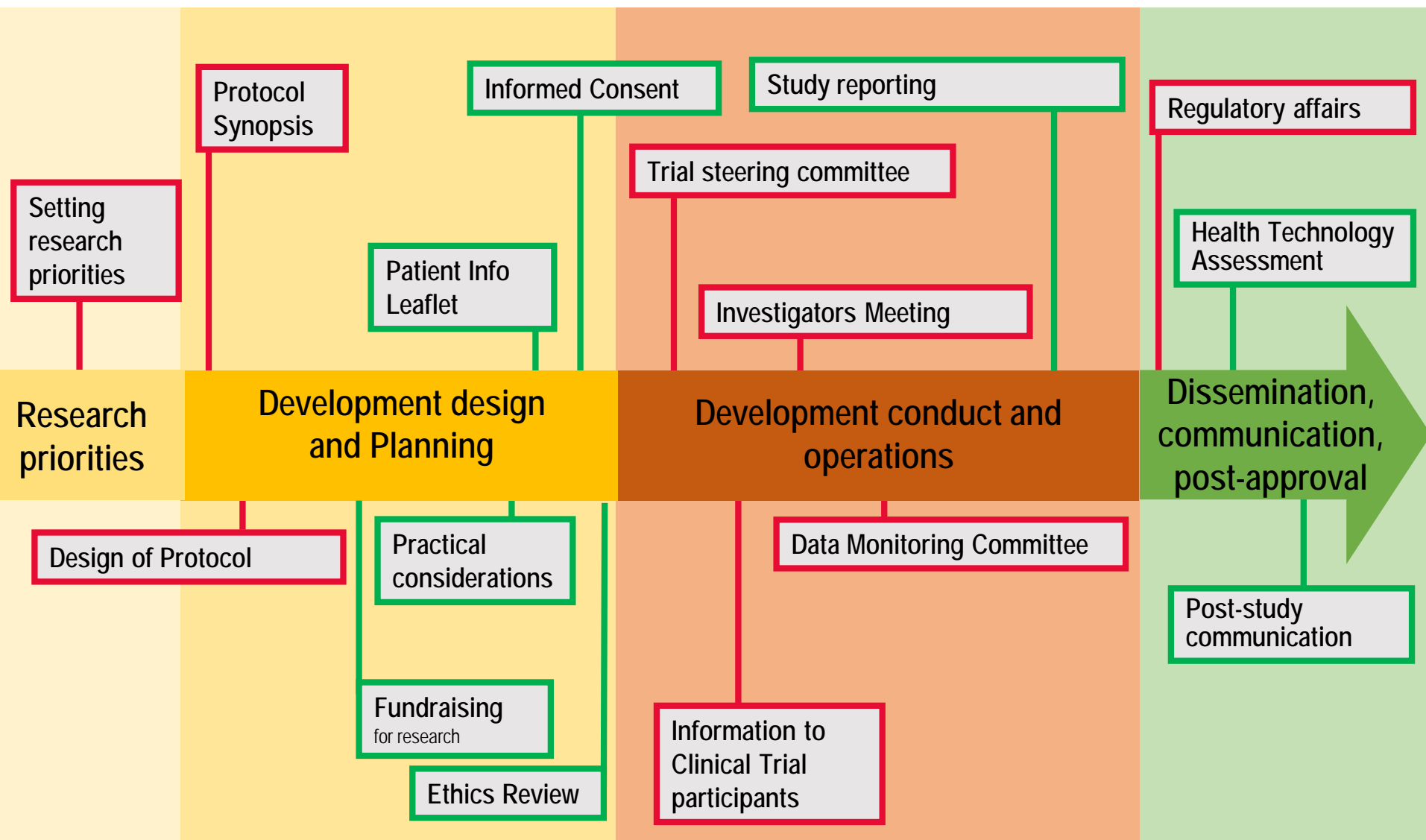


Patient Engagement in Industry

Opportunities for patient engagement / involvement
in industry

General overview

Practical “Roadmap” on patient involvement in Research and Development (R&D)



Level of expertise in the disease area required:

high
medium

Practical “Roadmap” on patient involvement in R&D:

Unique insights of “expert patients” along the whole R&D life cycle

R&D Process

Setting research priorities

- Gap analysis
- Early horizon scanning
- Matching unmet patients’ needs with intended research outcomes
- Defining patient-relevant **added value** and **patient-relevant outcomes**

Development design and planning

- **Protocol design and synopsis:**
 - relevant endpoints,
 - in-/exclusion criteria
 - target population,
 - diagnostic procedures
 - patient-reported outcome / quality of life measures,
 - risk/benefit balance
 - cross-over,
 - ethical issues,
 - mobility issues,
 - data protection
- **Fundraising**
- **Informed consent and patient information:** content, visual design, readability, language
- **Ethics Review**

Development conduct and operations

- **Investigators Meeting:** patient perspective on trial, recruitment, challenges, opportunities, can trigger amendments
- **Trial Steering Committee and Data Monitoring Committee:** e.g. for risk/benefit, drop-out issues, amendments
- **Information to participants:** protocol amendments, new safety information
- Improving **patient access to trials**

Dissemination, communication, post-approval

- **Regulatory affairs:** EPAR summaries, package leaflets, updated safety communications
- Creation of **lay summaries** (as required by EU CTR)
- Contribution to **publications and dissemination of research results** to patient community and professional communities
- **Health Technology Assessment:** assessment of value, patient-relevant outcomes, priorities



Patient Engagement in Industry

Where are we with the implementation?

Preliminary findings from a DIA/Tufts University study prepared during the past months and presented at the DIA Annual Europe meeting 2016

DIA-Tufts University Study 2016 Preliminary Findings – Views on Patient Centricity

“Our organization is striving to put the patient at the center of everything we do, especially in R&D. We are working with patient organizations to integrate the patient’s voice into clinical development and clinical trials.”

‘Very Patient Centric’

3

‘Somewhat Patient Centric’

13

“There are pockets of interest and activity but this is not uniform.”

“The current patient centric approach is fragmented and inconsistently applied.”

‘Not Very Patient Centric’

4

“Timelines don’t always allow for us to be as patient centric as we want.”

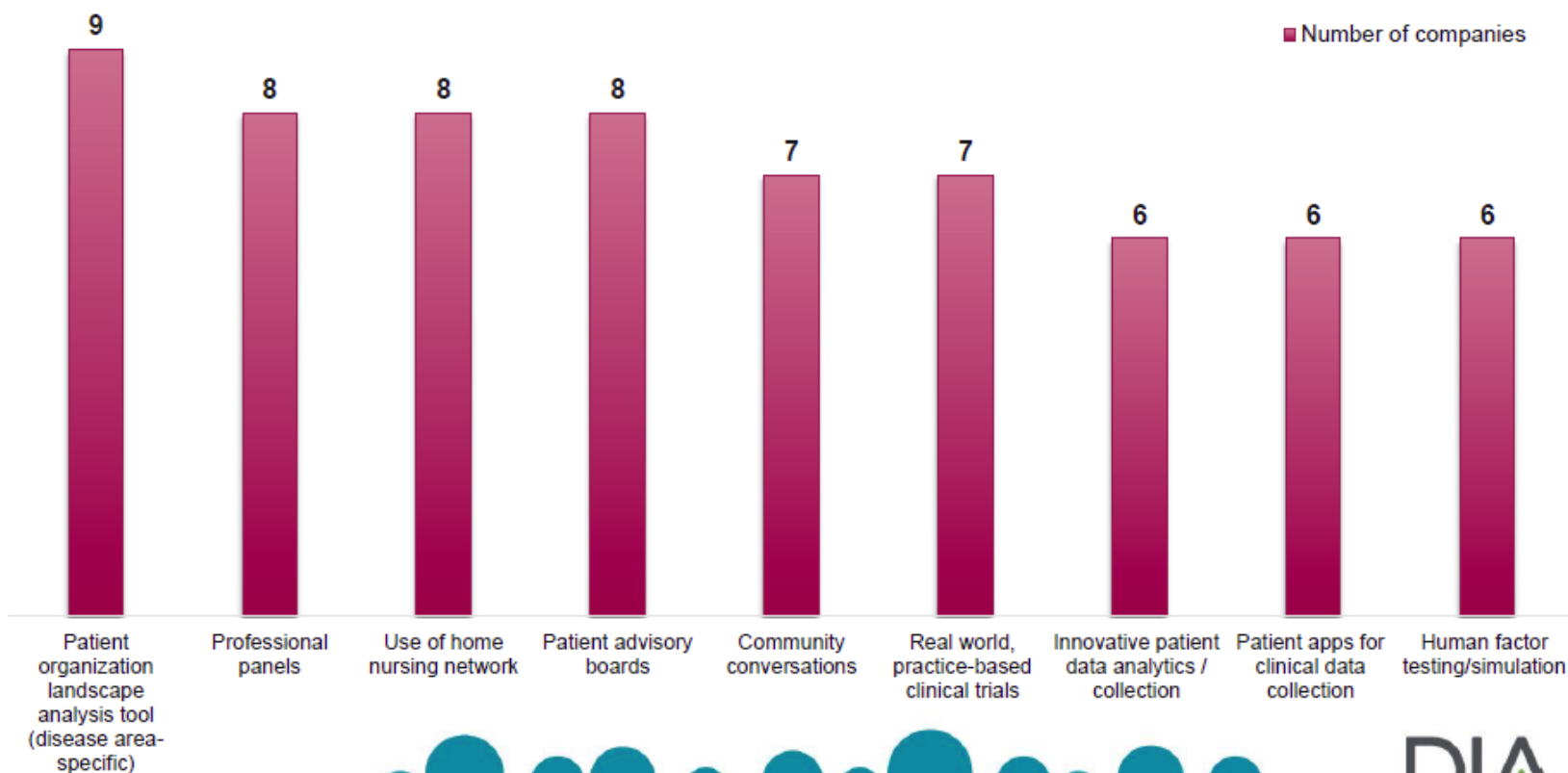
“At the moment, our activities are more scattered and embedded in various functions.”

Number of companies
N=20 companies



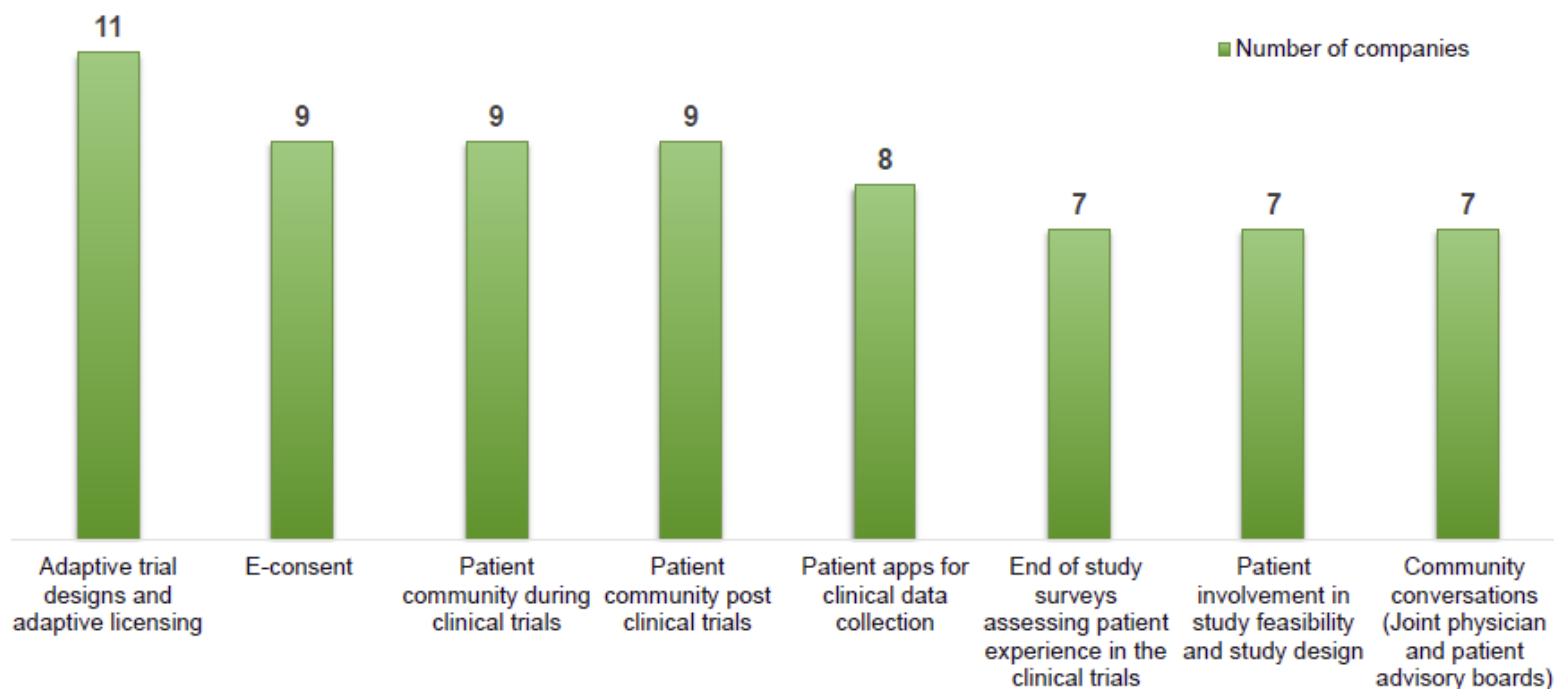
DIA-Tufts University Study 2016 Preliminary Findings – Patient Centric Initiatives Highlights

The most implemented initiative was a **patient organization landscape analysis tool**.



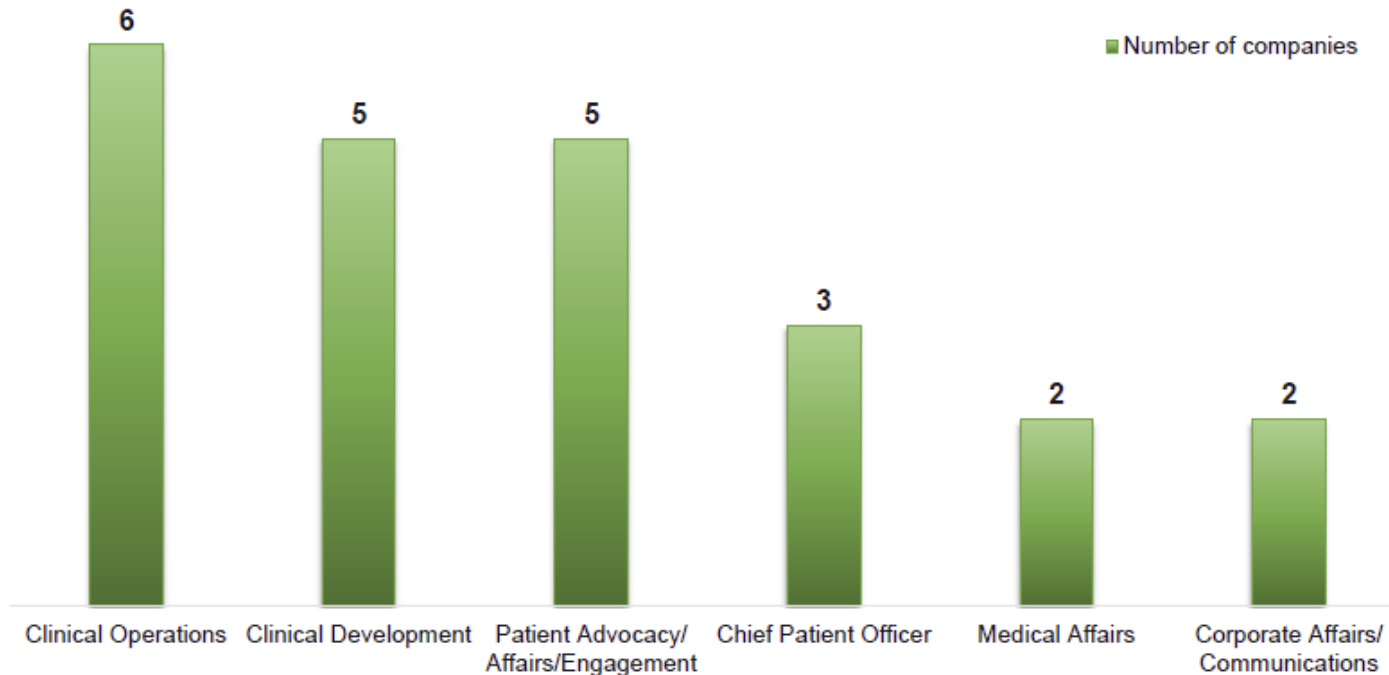
DIA-Tufts University Study 2016 Preliminary Findings – Patient Centric Initiatives Highlights

The **top planned initiatives** were adaptive trial designs and adaptive licensing.



DIA-Tufts University Study 2016 Preliminary Findings – Management practices and models for patient centricity initiatives

Patient Engagement Functions Sit in **Clinical Operations**



DIA-Tufts University Study 2016 Preliminary Findings – Management practices and models for patient centricity initiatives

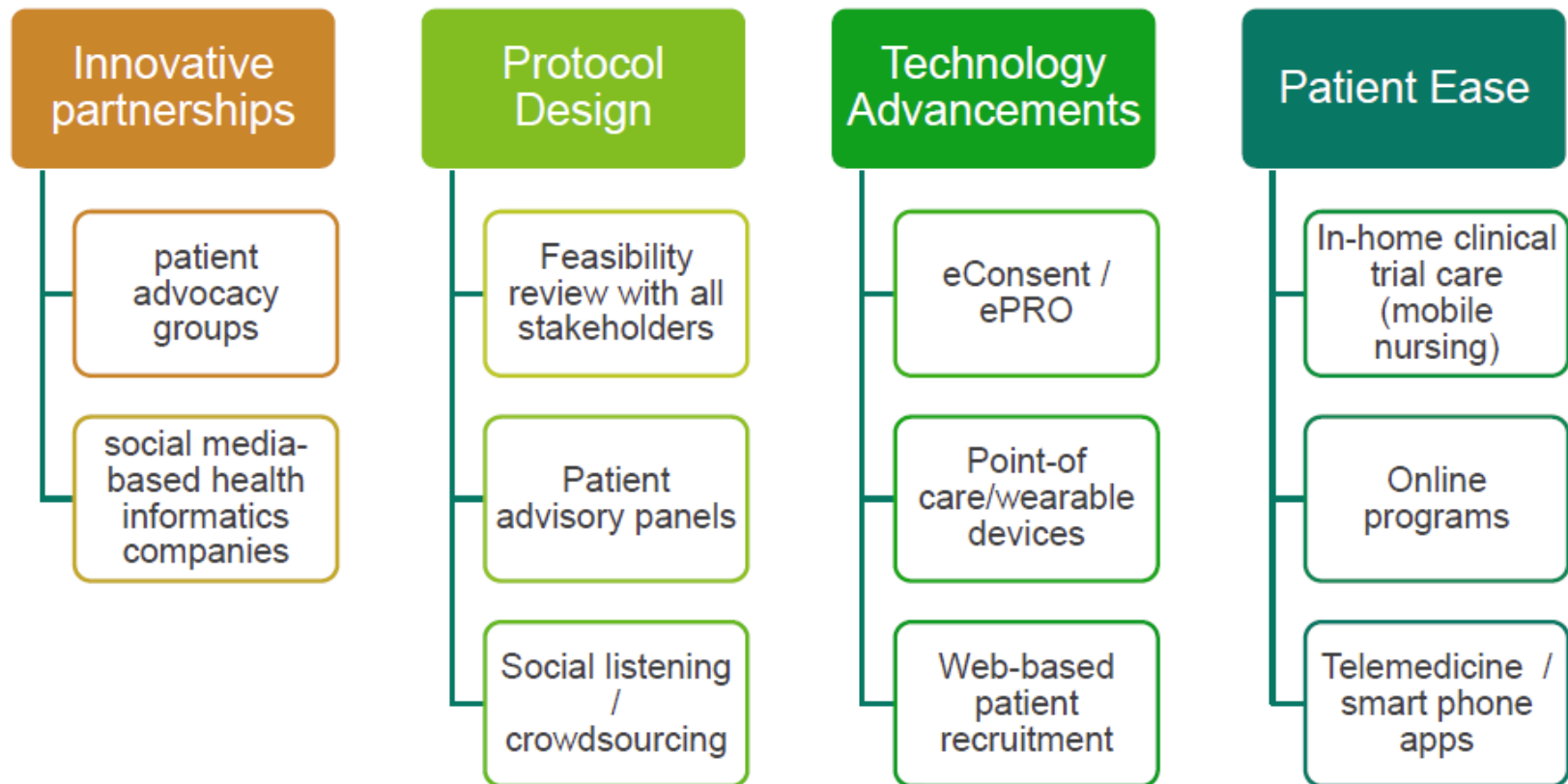
Corporate **drivers** to create a patient engagement function:

- ▶ **Company commitment** to demonstrate patient-centric culture (14/20)
- ▶ Part of **overall organization strategy** (11/20)
- ▶ Benefits **specific activity**; e.g., clinical trial design (9/20)
- ▶ Other comments:
 - “Fits the vision of personalized medicine”
 - “Commitment to patients”



DIA-Tufts University Study 2016 Preliminary Findings – Management practices and models for patient centricity initiatives

Categorizing Initiatives



DIA-Tufts University Study 2016 Preliminary Findings – Management practices and models for patient centricity initiatives

New Questions

Many! To be explored in the in-depth interviews:

- ▶ When multiple functions engage patients, do they communicate/coordinate? If so, how?
- ▶ How does the role engage internally to ensure the patient voice is integrated into an organization's culture?
- ▶ What key operational changes have resulted from patient-centric initiatives/focus?
- ▶ Are adequate resources allocated to patient-centric functions? What additional resources would be beneficial?



DIA-Tufts University Study 2016 Preliminary Findings – Management practices and models for patient centricity initiatives

Guidance is limited so far

- ▶ Some guidance from FDA *and EMA*
 - Devices ahead of drugs
- ▶ Patient organizations driving patient-centricity
 - Proposed guidance documents from patient organizations (e.g., PPMD, HIV/AIDS, Chronic Fatigue)
- ▶ Umbrella organizations developing toolkits and resources
 - CTTI, EUPATI, Genetic Alliance/NHC, Global Genes, PCORI
- ▶ Rare diseases may be ahead of major diseases
 - Challenging assumptions in order to gain efficiencies





Patient Engagement in Industry

What are the main challenges?

Why are we here today?

Challenges for patient involvement (in R&D)

- Concerns about patient independence when engaging with industry/regulatory authorities
- Need for comprehensive guidance/framework addressing **all** stakeholders
 - Adequate safeguards for patients and any experts to protect their integrity and credibility, avoiding ever tighter regulation
- Infrastructure for “matchmaking” between industry opportunities for patient involvement and available/willing patients (advocates)
- No consolidated approach for patient engagement in industry R&D
- No metrics for assessing the impact of patient involvement in R&D
- Too few offers of R&D education, needed for patient empowerment
- Communication to the public, policy makers, media on value of patient involvement in R&D



Patient Engagement in Industry

Let's work together on joint solutions
for making patient engagement
in all parts of medicines R&D
and beyond a reality

Thank you for your attention!

Agenda



10:00	Setting the scene – patients, regulators and industry perspective	
11:30	Parallel work stream: INDUSTRY How industry processes and “actual implementation” has changed to involve patients in R&D: Cases and discussion	Parallel work stream: REGULATORS Sharing how EMA and NCAs approach and/or implement patient involvement in regulatory authorities’ processes: Cases and discussion
(12:30 Lunch)		
14:30	Report from the two work streams	
15:00	Discussion How does that all fit together? Risks, independence, mishaps, educational needs, guidances	
16:00		