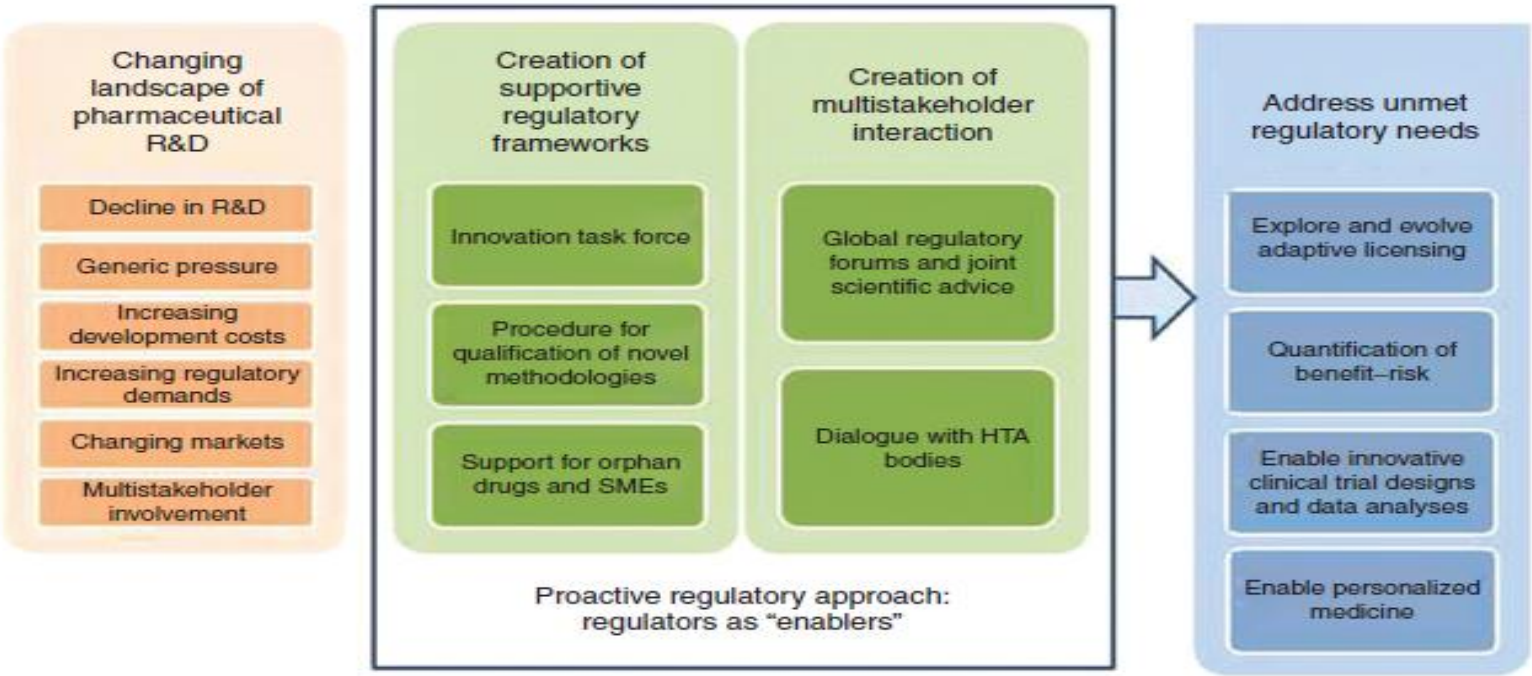
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Early interactions on innovation at EMA (ITF)

Session 2: Early interactions with regulators to support innovative medicines and technologies

Presented by: Falk Ehmann, Science & Innovation Support (EMA)

Regulators became gatekeepers and enablers…



Clinical pharmacology & Therapeutics; Advance online publication 3 April 2013. doi:10.1038/clpt.2013.14 ; F Ehmann, M Papaluca Amati, T Salmonson, M Posch, S Vamvakas, R Hemmings, HG Eichler and CK Schneider

Innovation Task Force (ITF)

Multidisciplinary platform

for preparatory dialogue and orientation on

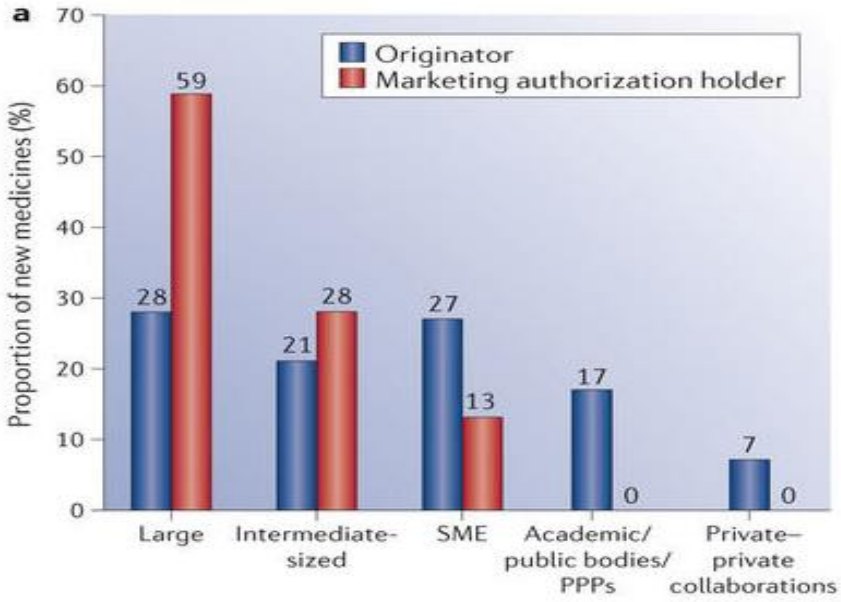
innovative methods, technologies and medicines

ITF objectives (ASAP):

- **Assist Knowledge exchange** on innovative strategies involving **regulatory network**
- **Support drug development** via early informal dialogue on
  - **Scientific, legal and regulatory** issues
  - Products, **methodologies and technologies**
- **Address the impact of emerging therapies and technologies** on current regulatory system
- **Preparing for formal procedures**

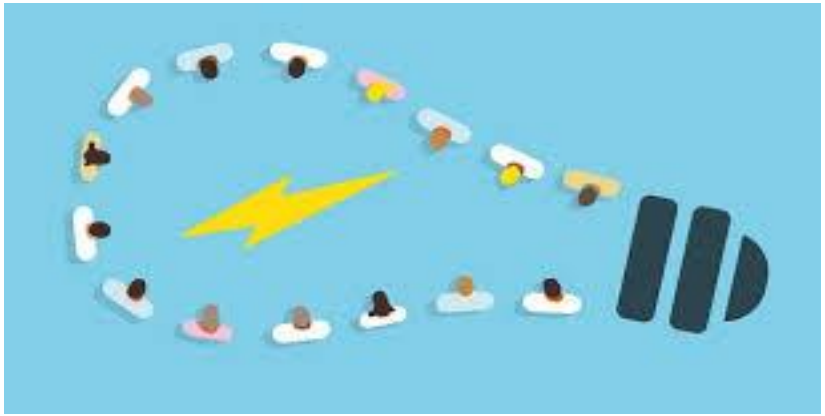
Users of the Innovation Task Force

Originator and the marketing authorization holder for 94 approved products evaluated, divided according to organization type

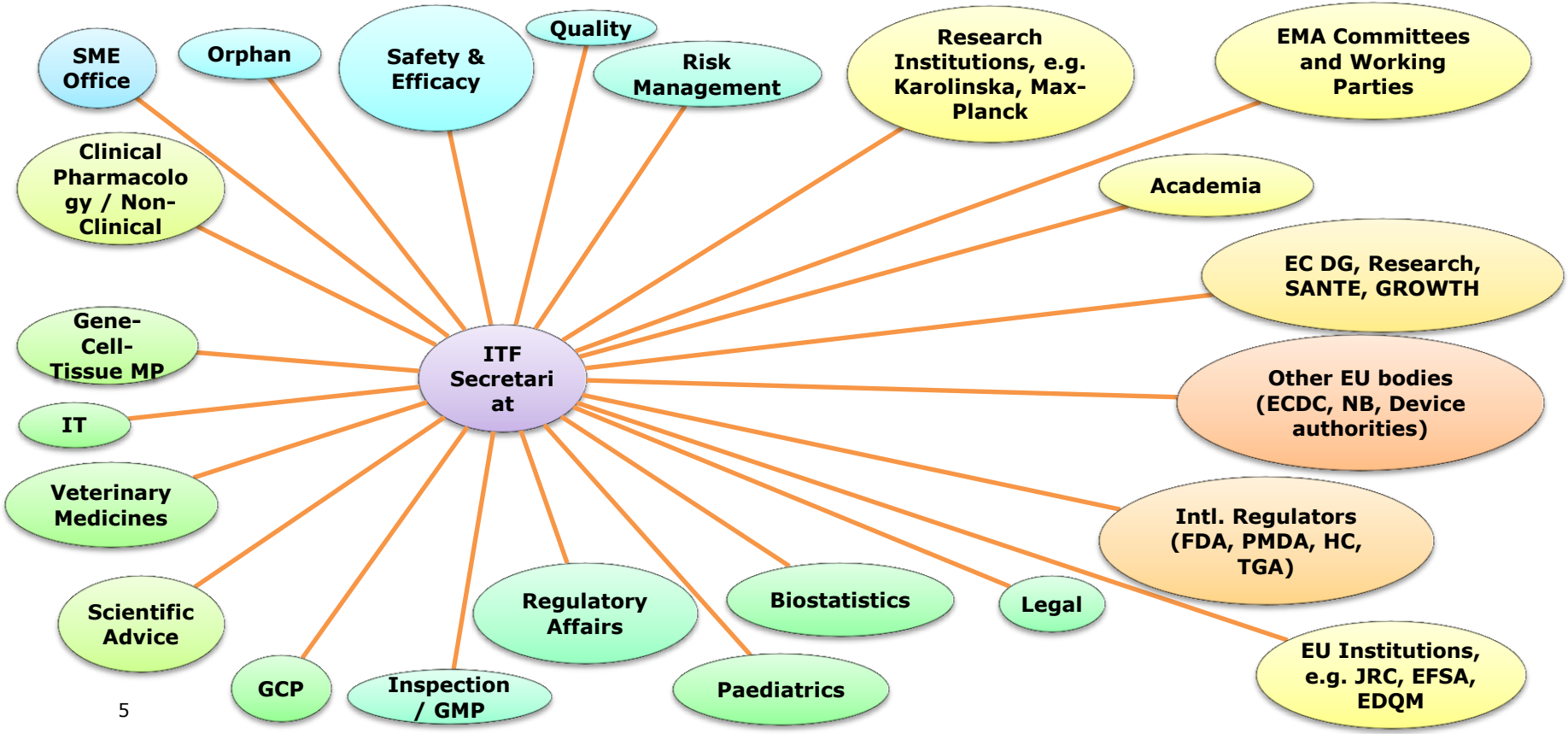


Regulatory watch: Where do new medicines originate from in the EU? Nature Reviews Drug Discovery Volume: 13, Pages: 92–93; Published online 31 January 2014

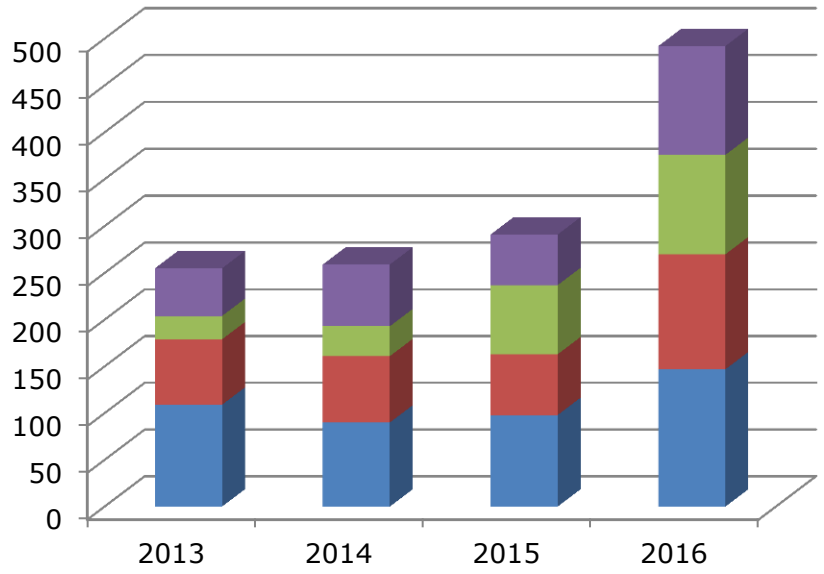
Since 2016 the ITF supports informal meetings with research and public-private funded consortia, e.g. IMI, HZ 2020, FP7 etc.



Multidisciplinary ITF resources (internal and external):



Involvement in ITF Briefing Meetings (internal and external):



Year of meetings	2013	2014	2015	2016
Number of meetings	23	27	33	41
ITF attendees	51	66	54	116
EMA attendees (non ITF)	25	32	74	106
WP experts from EU Regulatory Network	70	71	65	123
Industry attendees	109	90	98	147
Total	255	259	291	492

Emerging Technologies:

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Novel treatment technologies:

- Genome editing
- 3D printing of viable cells
- Externally activated products (magnetic / photo)
- Microbiomics
- M-health and E-health (Apps)
- Disease modelling
- Treatment algorithms (CT → software)
- Epigenetics

Novel manufacturing technologies:

- Nanotechnology
- Biomaterials
- Continuous manufacturing / QbD

Innovative products in clinical development:

- **ATMPs**
  - Gene edited modified cells
  - Stem cell based (facilitated by novel stem cell sources)
- **Nanotechnology incorporating products**
  - Guidance in 4 areas: Block Co-Polymer Micelles, Iron-oxide, Liposomal formulations, Coating
- **Novel "complex" products (Hybrids**)
  - Complex synthetic oligonucleotides and peptides (mixtures) [Allergens / "*Cancer-Imunoth.*"]
  - Phages (not novel but revival)
  - Multifunctional recombinant biologicals - including borderline
  - Bi/multi-functional monoclonal antibodies and conjugated monoclonal abs / chemicals,
- **Biomaterials**
  - Novel polymers

Impact of Innovation Task Force:

92 ITF Briefing meetings organised between 2014 – 2016, of which **80%** were submitted by **academia, SMEs and consortia** (ITF support focus)

- **30%** of applicants consider applying a **formal scientific advice** request
- **11%** consider **Qualification of methodology** (e.g. Biomarker qualification)
- **15% are Advanced Therapies** (Gene, Cell, Tissue engineered products)
- 14% consider seeking EU Orphan Drug designation (rare diseases)
- 20% consider interaction with the EMA Paediatric Committee (PDCO)
- **10% consider Marketing Authorisation Application** within foreseeable future

ITF Impact:

ITF interactions, collaborations, meetings and minutes

- ITF-BM **Tracking database** as constant tracking tool
- **Intelligence gathering including stakeholder consultation**

- Monthly briefing and **feed-back** provided **to Committees**
- **Trainings organised (internal and external)**
- **Awareness sessions broadcasted** via EU-NTC
- Recommendations for **workshops, expert meetings**
- Recommendations for **Drafting guidance**
- **Input in Horizon Scanning and EU Innovation Network**

Recently identified priorities and follow-up actions:

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Identified Topic / area:	Follow-Up actions:
•Companion diagnostic vs. complementary diagnostic	→Integrate concept in PGWP Co-development CP (pub 2Q17)
•Probiotic Bacteria (LPB); Classification / Usage of live GMO as delivery vehicle	→EFSA, EC and EU-IN interaction / awareness session
•Organoids to better mimic and predict Non-Clinical testing (3Rs)	→EMA workshop in October 2017
•Gene editing methods and its implications	→PGWP / CAT expert meeting on Genome Editing Technologies (Oct 2017) and ATMP matrix action plan

ITF – EU-Innovation Network collaboration:

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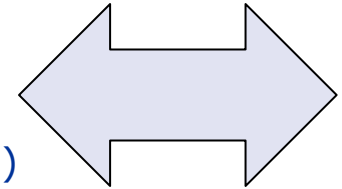
ITF

- **Bedside manufacturing**
  - bring the (individualised) product to the patient,
  - technical integration
  - cont. manufacturing / QbD
- **Reg. framework** for "personalised" **Medicines**
  - N=1 trials (orphan experience)
  - treatment algorithms
  - Conditions vs. MoA
  - Modelling and Simulation / Extrapolation
- **eHealth**
  - Health Apps, (claims made)
  - electronic data collection / processing in CTs / e-consent
  - Big data



EU-Innovation Network

- **Borderline products incorporating novel technologies**
  - Devices / Cosmetic / Food → Def. MoA Biomaterials
  - Demarcation towards cell, tissue and blood regulation
  - Combination products / principle MoA
  - Nanotechnology (PMDA guidance on siRNA products)
- **"Valley of death"** during drug development
  - Non Clinical dev. → **First in Man CT** → Phase I-III design

Take home messages

- Regulators became gatekeepers and enablers
- The European Regulatory Network is open to discuss scientific, regulatory and technical aspects of innovative developments
- The ITF is the Regulator's tool for informal early engagement and feedback

Further information

See: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000334.jsp&mid=WC0b01ac05800ba1d9

Contact us at: ITFsecretariat@ema.europa.eu

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