European Federation of Pharmaceutical Industries and Associations

Think Big reflection process

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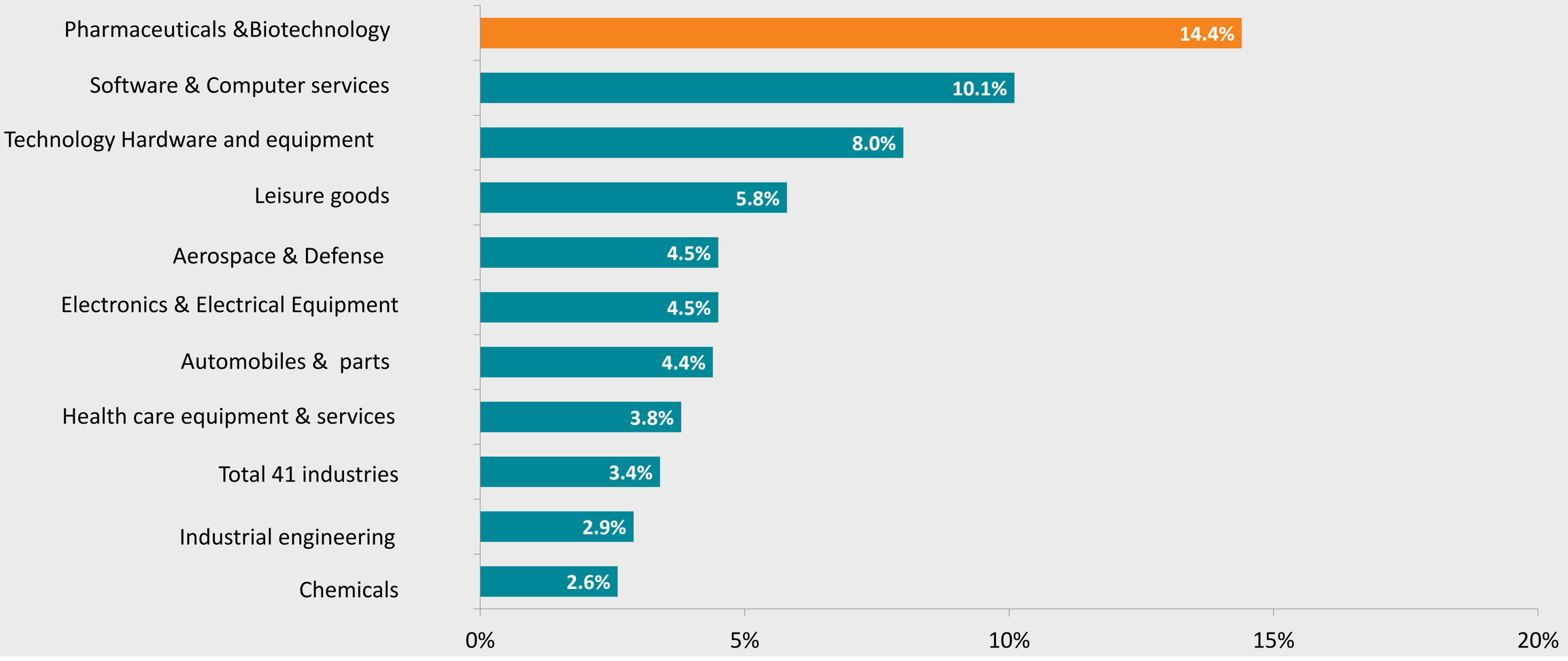
Pharma industry – background

The pharmaceutical industry is the sector with the highest R&D intensity

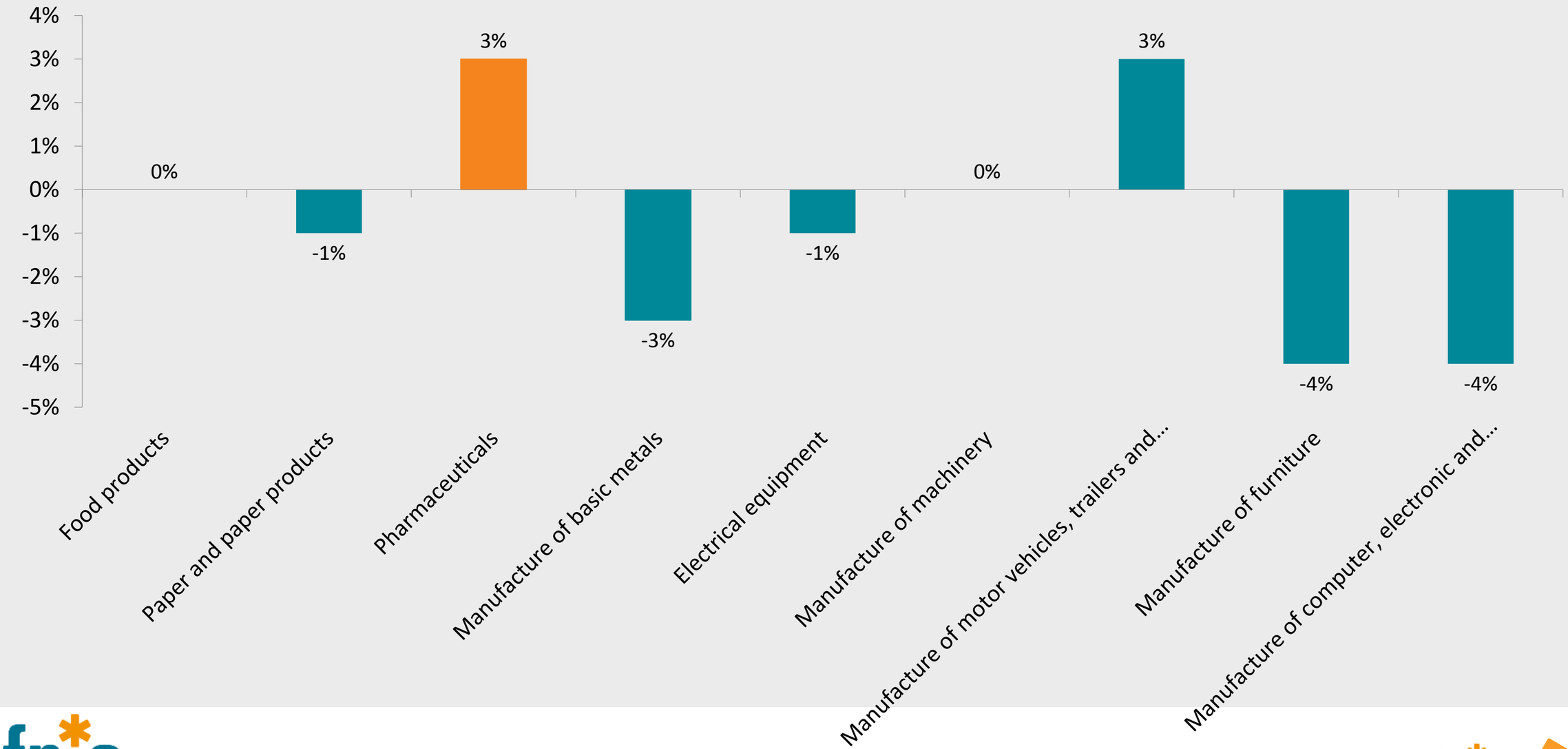
Ranking of industrial sectors by overall R&D intensity (as percentage of net sales, 2014)



Note: data relate to the top 2,500 companies with registered offices in the EU (608), Japan (360), The USA (829) and the Rest of the World (703), ranked by total worldwide R&D investment (with R&D investment above €17.9M)
Source: The 2015 EU Industrial R&D Investment scoreboard, European Commission, JRC/ DG RTD.

Employment in the pharmaceutical industry has proven to be more resilient than many other sectors in the EU

Percentage change in employment in selected industries in EU28 (2012-2014)



Source: Eurostat database on employment by NACE2 sectors, accessed in January 2016.

An upsurge in innovation and emergence of new healthcare technologies has the potential to reshape patient care and disease

Why now?

1. **Rapid developments in the fields of biology, biotechnology and medicine**
2. **Refinement of technology through incremental gains following trial and error**
3. **Advent of big data and predictive analytics**
4. **Increased R&D expenditure (rose by >50% between 2000-2011*)**
5. **Patient-centric treatment and genetic information are redefining how we use and develop tools**

Innovative new health technologies

Radical innovation is already here – immunotherapies have significantly increased the life expectancy of melanoma patients and nucleotide analogs have provided a cure for Hepatitis C

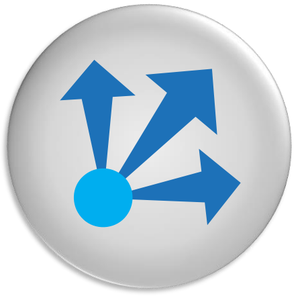
**www.oecd-library.org (see note section for full source)*

Over the next 5 years many, promising innovative product classes/technologies are expected to come to market

The illustrative subset below is a diverse mix of innovation expected to launch in approx. 5 years:

CAR-Ts	• CAR-T therapies are T-cells that have been genetically modified to express Chimeric Antigen Receptors (CARs) that target a tumor associated antigen allowing the T-cell to recognize and destroy tumor cells
Gene Therapy	• Gene therapy is when DNA is introduced into a patient to treat a genetic disease. The new DNA usually corrects or inserts a functioning copy of a mutated gene responsible for the disease
Cell therapy	• Cell therapy involves insertion of living cells into patients to replace / repair damaged tissue in order to substitute or facilitate improved organ / tissue functionality
Combination Regimens (Oncology)	• Combinations regimens promise to deliver superior outcomes vs. monotherapies by manipulating different mechanisms of action or multiple pathways across the tumour response cycle
Modifying Therapy (Alzheimer's)	• Disease modifying therapies in Alzheimer's seek to breakdown or inhibit the formation of β-Amyloid and/or tau protein plaques thought to cause the disease via alternative pathways
Antibacterial mAbs	• Antibacterial antibodies bind to and neutralize highly evolutionarily conserved pathogenic bacterial surface proteins or secreted toxins and activate the immune system to directly kill the bacteria

Championing upcoming innovation will pave way for further exciting innovation on the horizon with transformational promise



- **Disease understanding is growing** thanks to improved techniques and an ever increasing information base to draw upon, leading to personalised healthcare based upon patient genetics
- **Investing now** will both **drive improvements in the innovations** we are exploring and **encourage future investment in new innovation**

Driving Improvements in Current Innovation

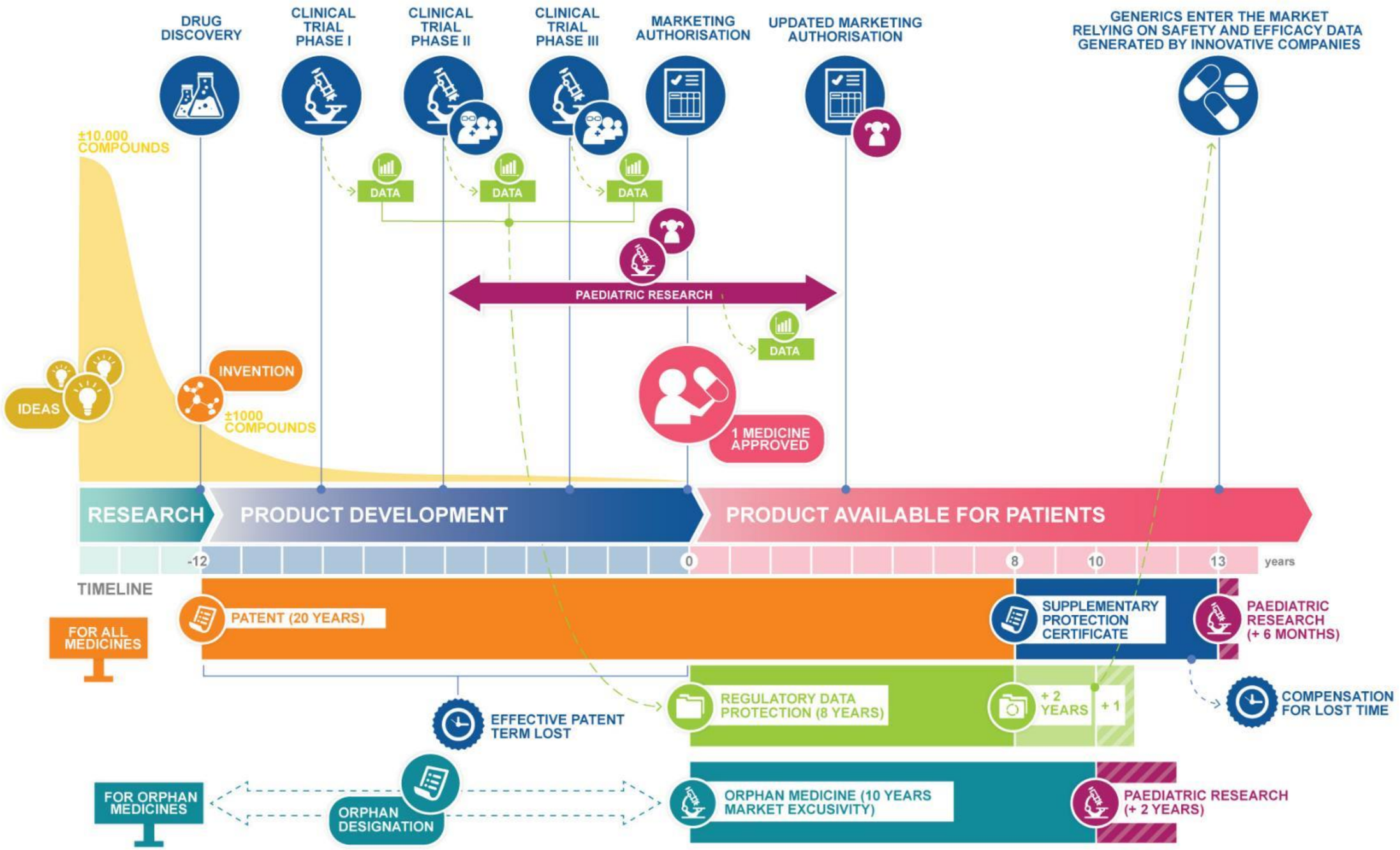
- **Personalised Treatment:** Advances in oncology will drive the trend towards personalized combinations of drugs based on patient genetics which **will increase survival**
- **Alzheimer's Diagnostic:** Fuelling the current drive to find a treatment for Alzheimer's will also mean that progress is made towards being able to **diagnose and treat patients before clinical symptoms appear**
- **Antibacterial Arsenal:** More research into mAbs will **increase the number of MDR infections that can be targeted** and help us understand how to **continue to stay one step ahead** of infectious disease

Encouraging Future Innovation

- **CRISPR: Gene-editing technology** is expected to be a **medical breakthrough** in a number of indications like **cancer, infections and muscular dystrophy**
- **Peptide Immunotherapy:** This **Antigen-based immunotherapy c**ould be an **optimal therapy** for **auto-immune disorders** as can be disease-specific and limit systemic side effects
- **Check-point Stimulators:** Combining their agonistic effect with other therapeutic strategies is expected to **deliver greater results in cancer patients**

ims consulting group

EUROPEAN PHARMACEUTICAL INCENTIVES FRAMEWORK



The Think Big Process

Portfolio for 2018 onwards

Think Big process – summary

- **Global Heads of R&D drive definition of key priority research areas that will make the best use of the remaining IMI2 public and private investment**

- **Commitments expected in 2018-2020:**
  - Public funding for grants for public partners: ca EUR 850 mln
  - In-kind investment by companies: ca EUR 900 mln

- **Four areas prioritised**
  - AMR
  - Immunology
  - Digital health
  - Modernisation of clinical trials

Fast-track idea generation and long-term planning

Kick off workshop and identification of 4 key priority areas

[Global Heads of R&D]

13 March – 15 May

Workshops to define the scope of 4 prioritised areas

[led by Head of R&D "sponsors": E. Zerhouni (Sanofi), J. Reed (Roche), P. Stoffels (Janssen), P. Vallance (GSK), M. Dolsten (Pfizer), V. Narasimhan (Novartis)]

23 June – 30 August

Development of topics in each area

30 June – 8 November

Consultation with IMI advisory bodies for ideas planned to be launched in 2018-2019

November onwards

4 priorities and first batch of ideas: Summary

Immunology	• Treatment of non-response and remission • Non-invasive molecular imaging of immune cells
Antimicrobial resistance	• Clinical trials networks • Accelerator of AMR R&D
Digital Health/Big Data	• Remote clinical trials • Biosensors/digital endpoints in clinical development
Modernisation of clinical trials and regulatory pathways	• Addressing the challenge of platform trials (Integrated Research Platforms)

First batch of early ideas: Objectives (1)

Immuno: Treatment of non-response and remission	Better control of immune related diseases. Improved patient management /personalized treatment by validation of predictive biomarkers for non-response and remission
Immuno: Non-invasive molecular imaging of immune cells	Generation and validation of in-vivo immuno-probes as non-invasive early indicators of efficacy and outcomes for multiple disorders including Cancer, RA, Asthma, IBD, MS, Alzheimer's, etc.
AMR: Clinical trials networks	Network to provide an expert & sustained capability for AB trials in Europe and creates a significant push incentive for investment in AMR
AMR: Accelerator of AMR R&D	Enhance overall industry/SME/academia success in AMR discovery and progress assets/programs

First batch of ideas: Objectives (2)

Digital: Remote clinical trials	Bringing clinical research to patient as a new paradigm for running clinical trials. Combined with the adoption of digital endpoints, the flexibility of patient follow-up during clinical trials could reduce working costs in centralized hospitals, increase the frequency of data collection, increase data quality amongst others.
Digital: Biosensors/digital endpoints in clinical trials	New digital endpoints that take advantage of novel biosensor technology to increase the accuracy of endpoint so that data can be included in the label and be used in the market to monitor real world value.
Clinical trials: Integrated Research Platforms	Platform trials are already delivering time and cost savings. Groups setting up platform trials face common challenges. We propose to (i) develop best practices and (ii) enable the set up of several IRPs in diseases of common interest.

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