Benefits of Healthcare Biotech changing lives, bringing hope
Biopharmaceutical Industry in numbers

• More than **350 million patients** have already benefited from approved medicines manufactured through biotechnology and gene technology*

• More than **650 new biotechnology medicines and vaccines** are currently being tested for more than 100 diseases, including cancer, diabetes, Alzheimer’s, AIDS, rare diseases*

• **2.5 million** is the number of childhood deaths prevented worldwide each year by immunisation**

• More than **20 vaccines** for infectious diseases have been developed – many of them for children**

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* Source: Biotechnology Research Continues to Bolster Arsenal Against Disease with 633 Medicines in Development. PhRMA Report, 2008

Red biotechnology products in development by disease category*

*Source: PhRMA, Medicines in Development: Biotechnology 2011 Report
The pharmaceutical sector directly and indirectly addresses many of the priorities of the EU Horizon 2020

<table>
<thead>
<tr>
<th>Objectives of the Europe 2020 and Horizon 2020 initiatives</th>
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<tbody>
<tr>
<td><strong>Europe 2020 priorities and targets</strong></td>
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<tr>
<td>* Smart growth</td>
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<tr>
<td>* Education</td>
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<td>* Research and innovation</td>
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<td><strong>Horizon 2020 goals</strong></td>
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<tr>
<td>* Strengthen the EU’s position in science</td>
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<tr>
<td><strong>Pharma’s Contribution</strong></td>
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<td>* High-quality research jobs, academic partnerships</td>
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<td>* Europe’s most research intensive manufacturing industry</td>
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<td>* Sustainable growth</td>
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<td>* Sustainable low-carbon economy</td>
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<td><strong>Inclusive growth</strong></td>
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<tr>
<td>* Job creation</td>
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<tr>
<td>* Poverty reduction</td>
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<td>* Address concerns including affordability, sustainability, security and ageing</td>
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<tr>
<td>* Creating jobs in manufacturing, with spill overs to other sectors</td>
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<tr>
<td>* Reduces causes of social exclusion and inequality</td>
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</table>

Poor health has long been recognised as a key driver of social and economic inequality.

### Employment and at-risk-of-poverty rates for disabled and non-disabled EU citizens

#### Employment rate
- **No disability**: 68%
- **With disability**: 44%

#### At-risk-of-poverty rate
- **No disability**: 14%
- **With disability**: 21%

- **EU citizens reporting a disability are between 1.8 and 3.2 times less likely to be employed.**
- **The same individuals are also between 1.3 and 2.7 times more likely to be at risk of poverty.**

Note: "Disability" defined as responding either "Yes, strongly limited" or "Yes, limited" to the question: "Does the respondent have limitations because of health problems in activities people usually do, for at least the last 5 months?"

In widespread diseases: Diabetes

First major biotech treatment breakthrough: Insulin (1978)
Recombinant DNA technology produced Insulin in bacteria cells
Avoided allergic reactions – previously harvested directly from animals (pigs & cows)
No shortages – allowed enough to be produced

And in rare & serious diseases:
Haemophilia
Gaucher Disease
Mucopolysaccharidosis I (MPS I; alpha-L-iduronidase deficiency)
Idiopathic pulmonary fibrosis
...
Personalised (Stratified) Medicine
An emerging trend – future reality?

Matching Patients and Therapy

- **Diagnostic test positive**
  - Treat patients likely to benefit from treatment

- **Diagnostic test negative**
  - Remove unresponsive patients and explore other therapeutic options

- **Diagnostic test negative and reveals potential adverse reactions**
  - Remove unresponsive patients and explore other therapeutic options
The Value of Prevention

How Vaccines have changed our world*

*CDC web site 2013
## Scorecard: Results presentation *

<table>
<thead>
<tr>
<th>Government budget item</th>
<th>Value</th>
<th>Incremental Fiscal impact</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medical cost-savings</td>
<td>Savings</td>
<td>€6,651,724</td>
</tr>
<tr>
<td>Productivity loss (social insurance)</td>
<td>Savings</td>
<td>€4,199,281</td>
</tr>
<tr>
<td>Prevented disability costs</td>
<td>Savings</td>
<td>€502,426</td>
</tr>
<tr>
<td>Gross discounted tax</td>
<td>Revenue</td>
<td>€537,394,410</td>
</tr>
<tr>
<td>Vaccination cost</td>
<td>Cost</td>
<td>€136,878,802</td>
</tr>
<tr>
<td>B/C ratio</td>
<td>Return on investment</td>
<td>€4.02</td>
</tr>
</tbody>
</table>

* Investment €1 in adult immunization has the capacity to generate over €4 of future economic revenue for government

* SAATI / GMAS
Healthcare biotech “Version 2.0” delivering sustainable outcomes...

BUT

“the...drug could potentially be very effective for a small percentage of patients”

“Unfortunately, no [ways] have yet been identified to help identify this small group of people”

Sir Andrew Dillon
NICE, 2011

1. Effect size is most important
   ✓ Requires strongest possible link between therapy and specific patient

2. Evidence in real life
   ✓ Key to value-based patient access are outcomes over time
A vision for Europe

Complexity of data to demonstrate value

<table>
<thead>
<tr>
<th>RCTs</th>
<th>Comparative data</th>
<th>Real world data &amp; modelling</th>
<th>Various</th>
</tr>
</thead>
<tbody>
<tr>
<td>CLINICAL EFFICACY &amp; SAFETY</td>
<td>ADDED THERAPEUTIC VALUE</td>
<td>HEALTH SYSTEM VALUE</td>
<td>SOCIETAL VALUE</td>
</tr>
</tbody>
</table>

- Regulators
- Regulators & Payers
- Payers, Providers & Patients
- Payers, Providers & Patients

Complexity of data to demonstrate value
**Clinical Trials**

- **Phase 1**
  - Number of Patients/Subjects: 20-100
  - Duration: 6 – 7 Years

- **Phase 2**
  - Number of Patients/Subjects: 100-500

- **Phase 3**
  - Number of Patients/Subjects: 1000-5000

**Efficacy** describes how a drug performs under conditions of clinical trials.

**Regulatory Review**

- **EMA Filing**
- **EMA Approval for Sale**
- **HTA Approval**
- **Negotiation for Reimbursement**
  - 27 member States

**Effectiveness** describes how a drug performs in everyday clinical practice.

2 – 5 Years

**“Pre and post-licensing technologies will need to be harnessed to bridge the efficacy–effectiveness gap”**

Factors affecting incentives to invest in R&D

**Pre-market factors**
- Further commitments to pre-competitive research in IMI and Horizon 2020
- New development pathways for targeted medicines
- Progressive approvals

**In-market factors**
- Value-based pricing
- The right early benefit assessment
- Early, but managed entry schemes for entering the market
- Overall budgets to reflect value of medicines to society
- Equity and solidarity in access to medicines

**SUPPLY**

**DEMAND**
Conclusions

**Biotechnology**
Has already changed the therapeutic landscape for many severe, life-threatening and diseases – rare & common
Identification of the underlying cause – not merely symptomatic treatment
Next steps – stratification in targeting where a treatment can really make a difference
Future – resetting the agenda: prevention, diagnosis, treatment and cure

**We therefore**
Are ready to tackle the new challenges for drug development and stand to demonstrate the value of our products together with all the partners involved in healthcare

**However**
Stronger interactions and partnership with all stakeholders throughout the development and approval process will be key to make new innovations a reality for patients