Rare Disease Research Forum

Challenges and Solutions

Support and Needs of Industry

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Why Rare Diseases?

Society

- Rare disease patient groups now getting attention
  - EU growth plan establishes health and healthcare, including rare diseases, as priority area
- Disease understanding improving
  - Genetics of disease
  - Pharmacogenomics: who might respond to what
- Authorities encouraging
  - Orphan drug act
  - Office of New Drugs (OND) Rare Diseases Program
    - "To facilitate and support the research, development, regulation and approval of drug and biological products for...rare disorders"
    - "Complement the work of FDA's Office of Orphan Product Development"
- Medical need is urgent

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Why Rare Diseases?

Industry

- Medical need is urgent
- Chosen strategy
  - Develop your own proprietary drugs in orphan and specialist diseases
    - Relatively lower risk
    - SOBi: using biologicals expertise for niche indications
- Drug Repurposing
  - Existing drug can be applied to rare disease when pathology clarified

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Challenges

- >6000 rare diseases
  - Only 200 approved therapies
- Very few common denominators
  - Large diversity within rare diseases
- Small populations
  - Patient accessbility
  - Clinical feasibility
- Clinical development endpoints
  - Biomarkers and surrogate markers
  - Natural history outcome often serious (life-threatening) or even unchartered

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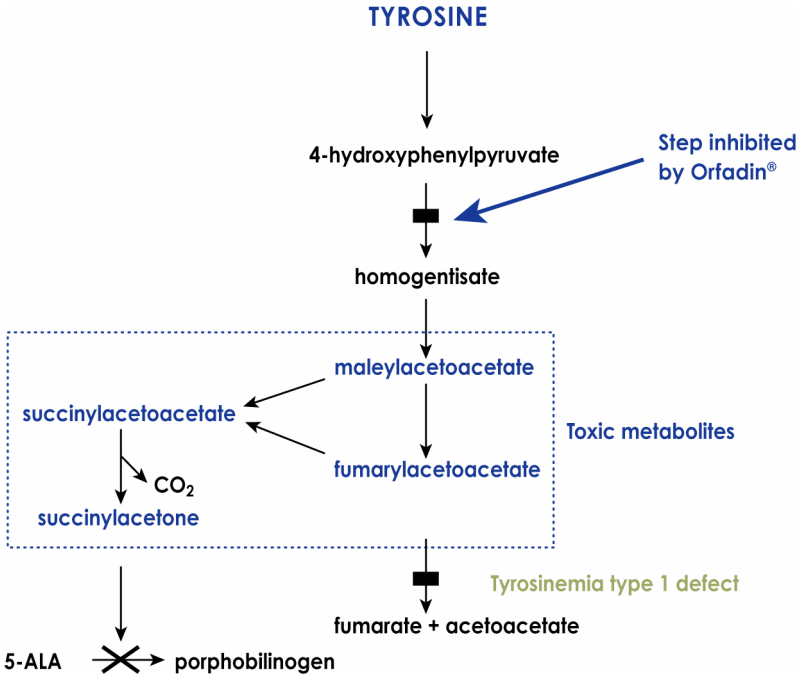
Drug Repurposing

Orfadin®

Bottlebrush plant (*Callistemon citrinus)*



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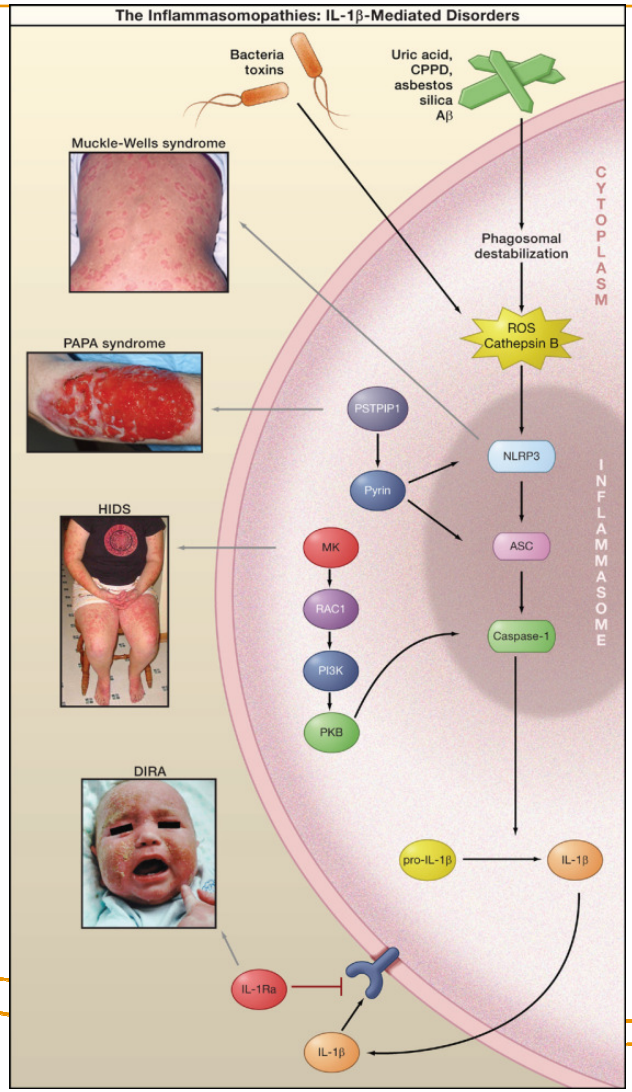
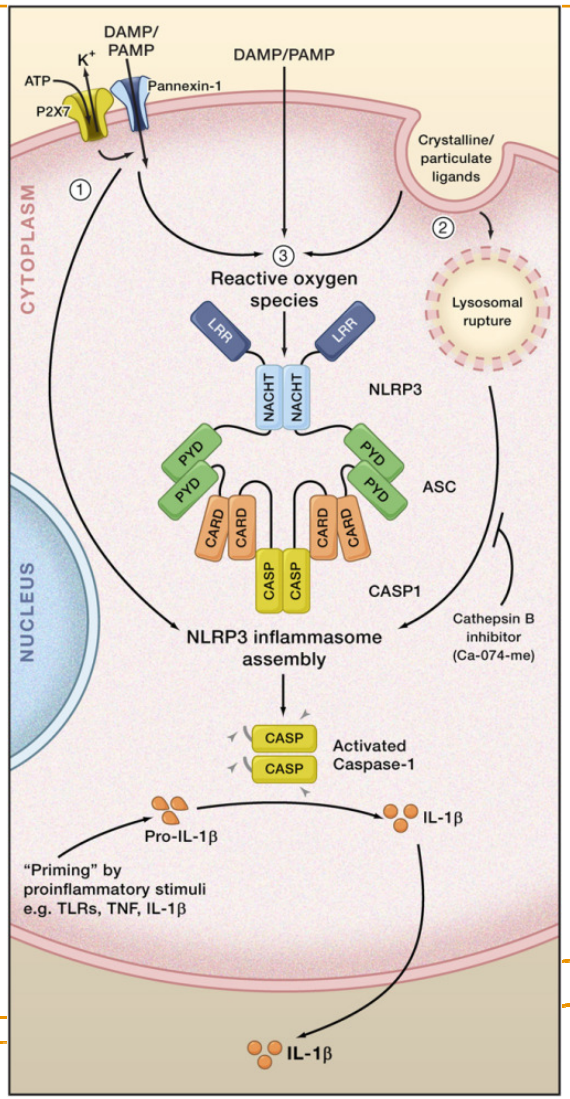
Drug Repurposing

Kineret®



Kastner et al 2010



Schroder & Tschopp (2010)

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Contributions of Industry to Academic Research (1)

- Compounds to be re-purposed
  - Laboratories for Chemical Biology at KI (LCBKI)
  - “Open Innovation” with Pfizer, Eli Lilly
- Clinical Development Expertise
  - Go straight into patients?
  - Toxicity data requirements?
  - Relationships with patient groups
  - Scientific advice with Authorities

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Contributions of Industry to Academic Research (2)

- Regulatory
  - Exploiting Orphan Drug legislation
    - When to apply
    - Impact on current product?
    - Help from Authorities
  - Navigating Paediatric Investigation Plan requirements in EU
    - Legislation since January 2007
    - *"aims at ensuring that the development of medicinal products that are potentially to be used for the paediatric population becomes an integral part of the development of medicinal products, integrated into the development programme for adults"* (Regulation (EC) No 1901/2006)
- Financial Assistance
  - To support the project or even the laboratory
    - Scientific risk must be manageable
    - Be aware that demands will be made on your research data transparency and your ability to publish at will

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Contributions of Academic Research to Industry

- Access to patients, clinical expertise, in depth understanding of the everyday life of the patient and relatives
- Pipeline Development
  - New understanding of pathologies
    - New hypotheses to test
  - Research models to test hypotheses and develop compounds
    - Especially animal models
- Networks of expertise and Centres of Excellence

Can Open Innovation be a way forwards between Academia & Industry?

In Stockholm for Rare Diseases

Closed Innovation

•When companies exploited science,governments didn't

•Immobile workforces

•No venture capital

Open Innovation

•Mobile workforces, high technology, the internet

•Venture capital

Seekers/Solvers

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