



BioPontis  
ALLIANCE  
FOR RARE DISEASES

## Biopontis Alliance for Rare Diseases:

a new collaborative model for bridging from  
discovery to treatment candidates

*Dr. Marlene Haffner & Dr. Erik Tambuyzer*

Vice-Chair & Chair of the Board

*ICORD 2014 – SESSION V*



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# Summary

- Discuss the challenges of developing medicines and diagnostics for rare diseases
- Introduce BioPontis Alliance for Rare Diseases as a new initiative and role for a philanthropic innovation joint venture model targeting rare diseases



and  
A VIABLE TREATMENT  
OR THERAPY

Patients are  
stranded  
between

THE PROMISE OF DISCOVERY



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Licensing hampered by  
misaligned incentives  
and interests

Complex R&D requires  
flexibly accessible,  
broad selection of  
technical capabilities

Industry requires well  
investigated, risk reduced  
compounds

Academic Research

Valley of Death

Industry

Discovery  
Science

Lead  
Discovery

Lead  
Optimization

Pre-clinical  
studies  
(to IND)

Clinical  
Development

Product  
Registration  
& Launch

Rare Disease Patient Organizations supporting  
therapy discovery, face the need to manage IP,  
learn applied product development and  
negotiation with partners



ACADEMIA &  
PATIENT  
ORGANIZATIONS

PHARMA &  
BIOTECH  
INDUSTRY

Our current system is  
not designed to address  
the suffering of people  
with rare disorders

GOVERNMENT  
REGULATIONS AND  
RESEARCH FUNDING



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## The Need for *Public-Private-Philanthropic Partnerships*

- Discovery and development processes change to meet the needs of patients (instead of focusing on creating “markets”)
- None of the players (industry, academia, regulators...) have all necessary knowledge
  - multidisciplinary collaboration to minimize size and cost of clinical trials
- Efficient model of “open innovation”: academia, clinicians and large & small companies to be partners in a win/win effort



Changing this development paradigm is the urgent mission of BioPontis  
We focus first on neurology...

A not-for-profit made up of industry and scientific experts who have found  
a way to improve the system

Product Development  
Scientists & Clinicians

CEO: Dr. Barbara  
Handelin, Genetic/Rare  
Disease diagnostics  
Genzyme

Board: Dr. Warren  
Strittmatter, retired Chief  
Dept Neurology Duke  
University

We are.....

Patient Organizations

Board: Susan Kahn,  
Exec Director, Tay -  
Sachs and Allied  
Diseases Association

FDA:

Board Vice Chair: Dr.  
Marlene Haffner, ex-  
Director FDA Office of  
Orphan Product  
Development

BioPharma industry

Board Chair: Dr. Erik  
Tambuyzer, ex SVP  
Genzyme, Founder CMI,  
ex Board chair EuropaBio

COO: Richard Basile, ex  
Management exec Bayer,  
Organon

Many professional  
volunteers in  
communications,  
fundraising,  
project evaluations,  
with pharma/biotech  
background...





We're bridging the gap between basic research  
and potential therapies



We form alliances with:

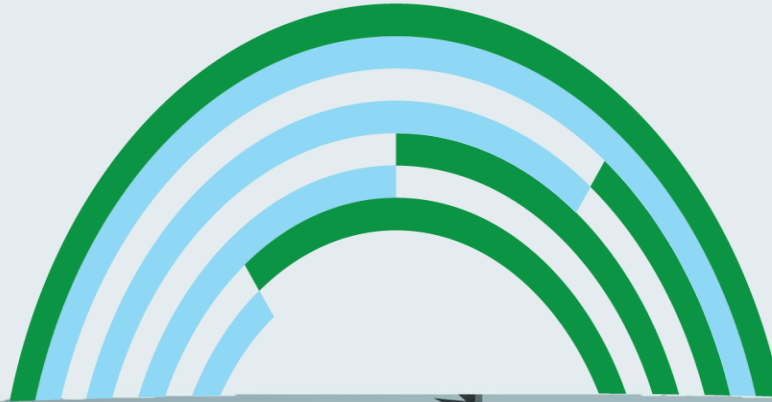
- Patient organizations
- Academic researchers
- Discovery laboratories
- Pharmaceutical & Biotech companies



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We're focused not just on identifying promising research, but on doing whatever it takes to turn those basic ideas into possible treatments that can be evaluated in people. We direct the discovery programs, and we do the heavy lifting necessary to move preclinical research forward.





It's no small task.

We recognize the challenges – and  
we're approaching them with a practical,  
results-focused frame of mind.



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## Partnering with Academia

Discovery Research  
Intellectual Property

University/Patient  
Organization  
Partner

Technology  
Evaluation  
Agreements

BioPontis Alliance  
For Rare Diseases

Master  
License  
Agreements

Discovery  
IP

Specific  
Technology  
License

R&D  
Project 1

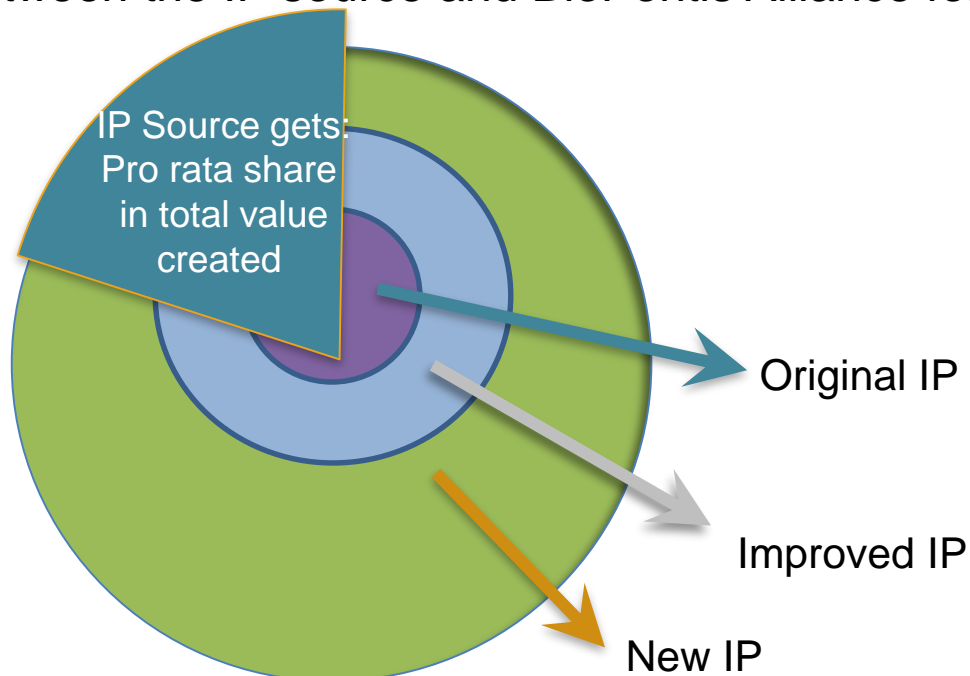
R&D  
Asset 2

R&D  
Asset 3

## Shared IP *and* economics model

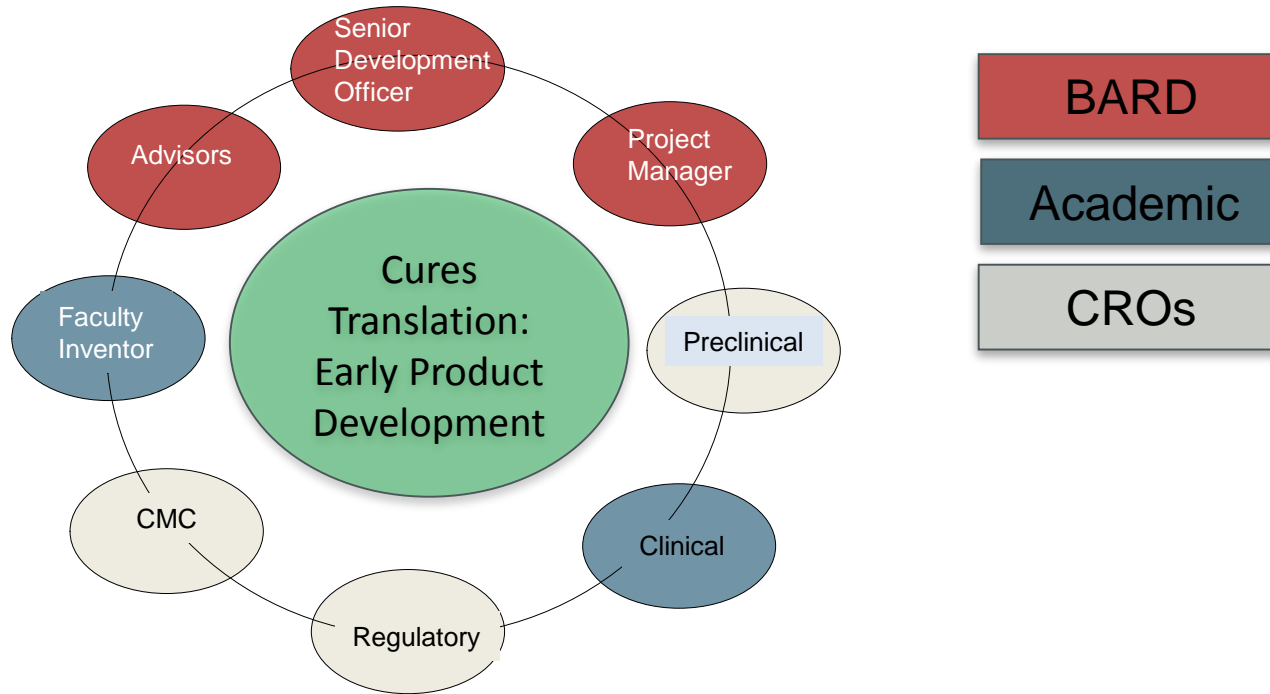
### Transformational Economic & IP Sharing

- Replaces upfront IP cost with shared exit IP value
- Value generated from the entire patent portfolio is shared between the IP source and BioPontis Alliance for Rare Diseases



- Removes concerns about IP work around
- Incentivizes all to maximize contributions toward success
- Promotes transparency and full collaboration

## How applied development happens: The Project Team



BARD creates a multidisciplinary team for each program bringing specific expertise around the inventing scientist that enables rigorous checking and advancing.



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One vision. One mission.  
*Deliver treatments to patients*

Joint Venture (JV) will house and protect existing/new IP

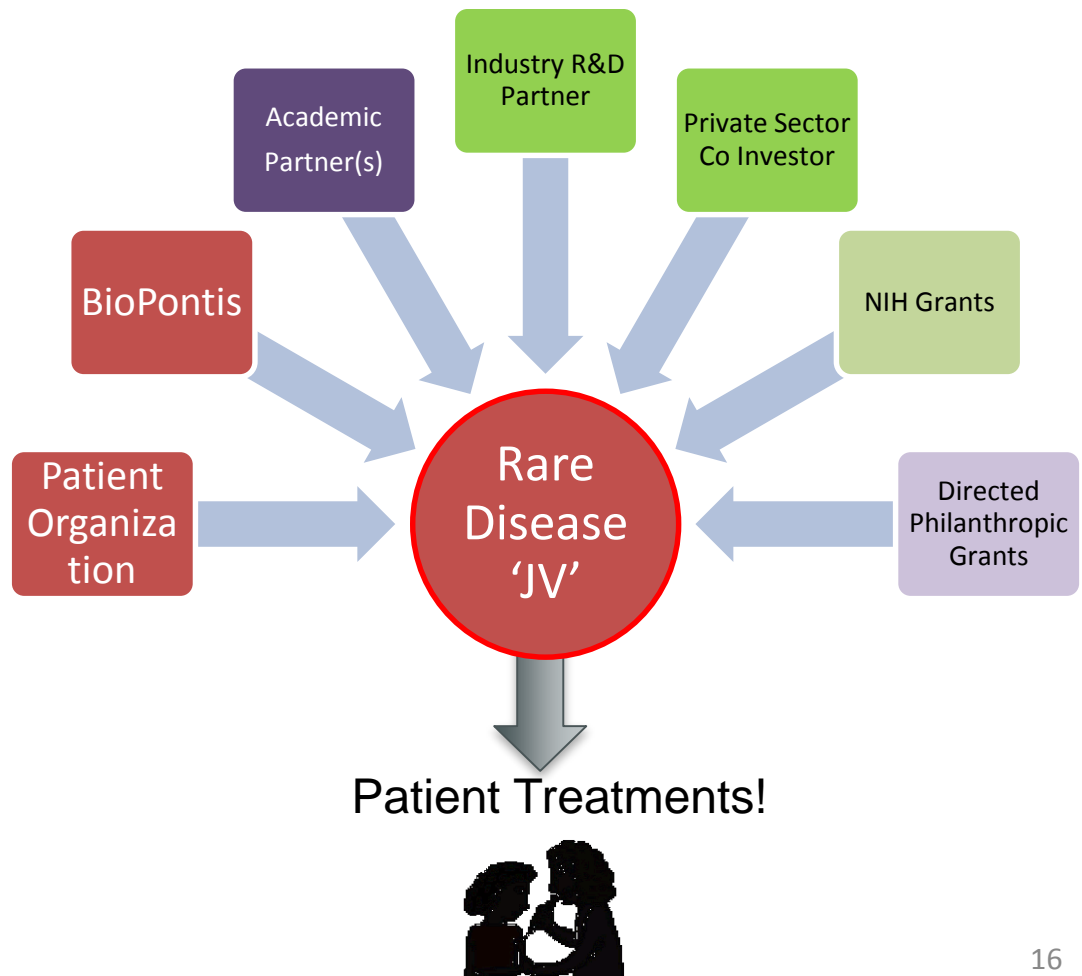
JV will enable in/out licensing for each treatment.

Academic partners incentivized through IP licensing terms.

JV will seek Private Sector 'co investment' as each project progresses.

IRS 501(c)3 compliance mandatory (US)

Exit vehicle to private sector – OR -  
JV can be non-profit pathway to product for patient treatment.



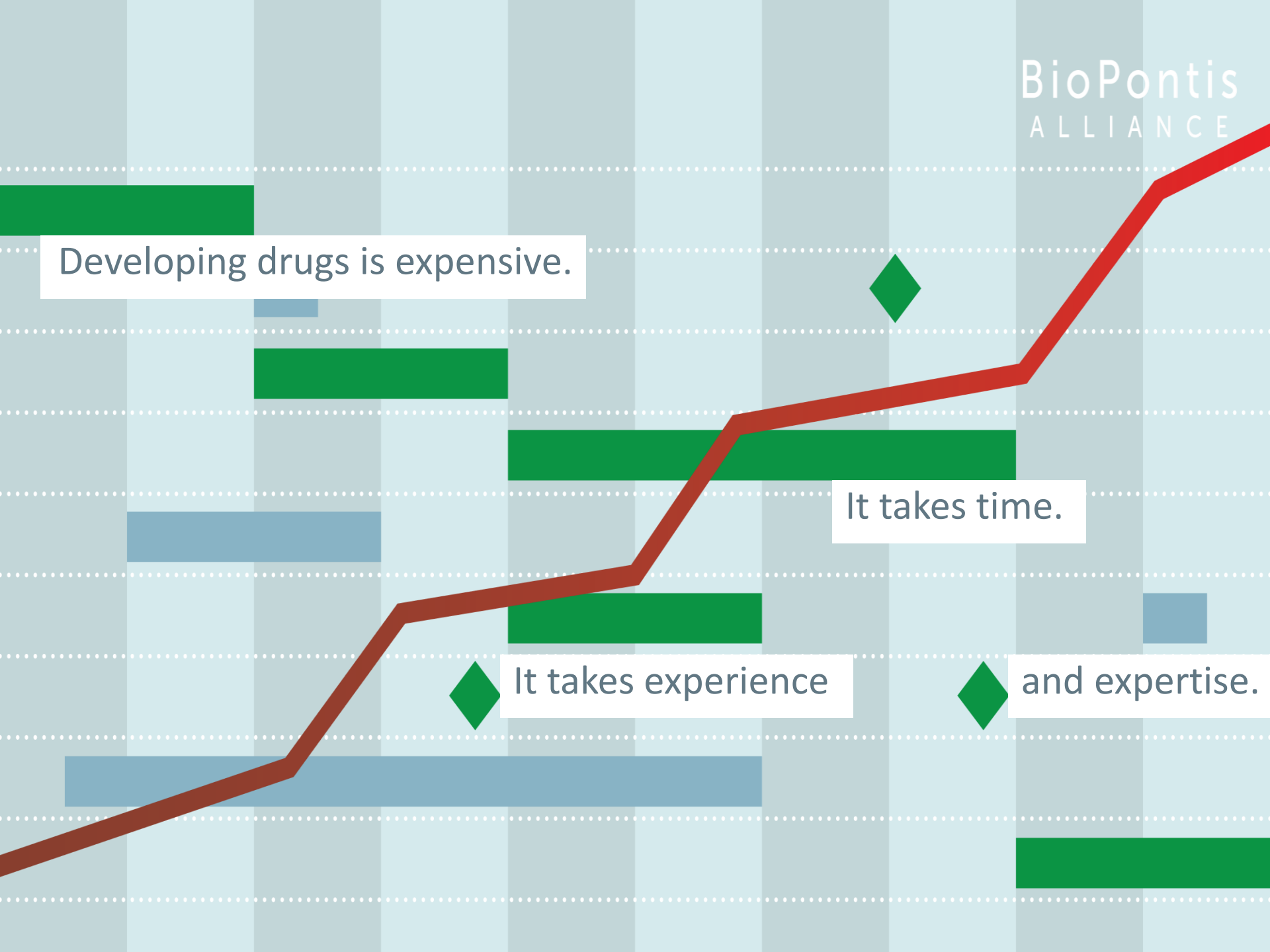


Developing drugs is expensive.

It takes time.

It takes experience

and expertise.



We are fundraising for \$2,000,000 to put our alliance into action

– to hit the “start” button on our bridge-building engine,  
and to enable partners to leverage your donation.

START HERE

Working together with those partners

– we will build that bridge all the way across.

## Our Asks

We look for:

- Committed volunteers for our fundraising, project evaluation and communications teams
- Rare disease therapy development projects (initially in neurology) for our pipeline
- Fundraising and donations to put our ideas into practice while we set up a European structure and ensure a global reach

Contact us:

- Barbara Handelin, CEO - [barbara.handelin@biopontisalliance.org](mailto:barbara.handelin@biopontisalliance.org)
- Richard Basile, COO – [richard.basile@biopontisalliance.org](mailto:richard.basile@biopontisalliance.org)
- Erik Tambuyzer, Chair – [erik@tambuyzer.be](mailto:erik@tambuyzer.be)
- Marlene Haffner, V-Chair – [marlene.haffner@biopontisalliance.org](mailto:marlene.haffner@biopontisalliance.org)

[www.biopontisalliance.org](http://www.biopontisalliance.org)

[www.biopontisalliance.org](http://www.biopontisalliance.org)

Mange tak

Dank je wel

**THANK  
YOU**

Merci beaucoup

Благодарам

Tänan teid

شكر

Dziekuje

Paldies

Grazie

Multumesc

धन्यवाद

Obrigado

Dankon al vi

Ευχαριστώ

Paljon kiitoksia

Tesekkür ederim

Toda raba

Nagyon köszönöm

ありがとうございます

Дуже дякую

Cảm ơn cô

Dakujem vám

Attyu

Hvala lijepa

Danke schön

谢谢

Tusen takk

Gracias

Благодаря

Dêkuji

Tack så mycket

Спасибо

Faleminderit