Open Science for Children’s Health

Open Science in Action:
M4K Pharma: An Experiment in Open Science Commercialization

Owen G. Roberts, CEO
M4K Pharma

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THE PROBLEM

• Science is outpacing our historical business models

• Monolithic diseases are now understood to be many sub diseases
  • Smaller patient populations
  • What were once considered to be large markets are now multiple small markets
  • Creating “Orphan Diseases” out of large market diseases

• Cost to develop therapies has not gone down in parallel
  • Cost to patient increasing to recover development costs

• Historical business model solution
  • Smaller market → charge a higher price
  • Effective therapies not being developed for small markets
WHY DO WE EXPECT ONE SIZE TO FIT ALL

Use appropriate business model in appropriate situations
AVOID THE ROUND PEG IN A SQUARE HOLE
THE PROPOSAL

• Open science can be used to reduce the cost of drug development and make small market drug development viable

• What is my definition of Open Science:

  *A commitment to rapid multilateral sharing of knowledge, results, data and materials without patent restrictions*

• Open science brings in funds from foundations, granting agencies, academic institutions and corporate “donations”

• Commercial opportunities remain:
  • If discovery/development costs are reduced → sustainable pricing can be lower
  • Priority Review Voucher for achieving registration of rare pediatric diseases
THE EXPERIMENT

• Select a disease that:
  • Represents an urgent medical need
  • Has a small or limited commercial market
  • Known genetic target
  • Our scientific partners have insight into the target
  • Favourable clinical path
THE EXPERIMENT CONT.

• Objective:

  • Access grants, foundations and corporate partners
  • Share (give AND receive) scientific insights
  • Aggressively promote open science
  • Find motivated partners
  • Retain IND data to gain international market exclusivity for out licencing
# Diffuse Intrinsic Pontine Glioma (DIPG)

## Paediatric Cancer: 5 Year Survival Rates

<table>
<thead>
<tr>
<th>Cancer Type</th>
<th>Survival Rate</th>
</tr>
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<tbody>
<tr>
<td>Retinoblastoma</td>
<td>97</td>
</tr>
<tr>
<td>Hodgkin lymphoma</td>
<td>97</td>
</tr>
<tr>
<td>Wilms tumor</td>
<td>90</td>
</tr>
<tr>
<td>Acute lymphocytic leukemia</td>
<td>89</td>
</tr>
<tr>
<td>Non-Hodgkin lymphoma</td>
<td>88</td>
</tr>
<tr>
<td>OVERALL</td>
<td>83</td>
</tr>
<tr>
<td>Neuroblastoma</td>
<td>79</td>
</tr>
<tr>
<td>Ewing sarcoma</td>
<td>75</td>
</tr>
<tr>
<td>Osteosarcoma</td>
<td>71</td>
</tr>
<tr>
<td>Rhabdomyosarcoma</td>
<td>68</td>
</tr>
<tr>
<td>Acute myelogenous leukemia</td>
<td>64</td>
</tr>
<tr>
<td>DIPG</td>
<td>&lt;1</td>
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The image shows a bar chart illustrating the 5-year survival rates for various pediatric cancers, with diffuse intrinsic pontine glioma (DIPG) having an extremely low survival rate of less than 1%.
WHY USE A CORPORATE STRUCTURE

• Corporate structure allows M4K pharma to apply for grants and foundation awards
• M4K will be a hub to “outsource” work to collaboration partners
  • Working with industry CROs and academic institutions
  • “Virtual” biotech model lowers costs
• Aggregating and aligning academic and industry collaborators to move along drug development path
• Creates a vehicle which can enter into agreements to sell and/or partner its assets
## M4K Pharma

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Open Science for Children’s Health

Original Patent Term - 20 years

- **US - New Clinical Studies**: 3+0.5 years
- **US - New Chemical Entities**: 5+0.5 years
- **US - Orphan Drugs**: 7+0.5 years
- **EU - Orphan Drugs**: 10+2 years
- **EU – New Chemical Entities**: 8+2+1 years
- **Canada – New Chemical Entities**: 6+2+0.5 years
- **Japan - Post-Marketing Surveillance**: 8 years

**Patent Term Restoration** (US) / **Supplemental Protection Certificate** (EU)

- **Orphan Market Protection** – no full or abbreviated submissions
- **Data Exclusivity** – no abbreviated submissions accepted
- **Market Exclusivity** – no abbreviated submissions approved
- **Post-Marketing Surveillance** – no generic applications permitted
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### Funding Sources

**Hits to Optimization:** Grants, Foundations, Corporate “donations”, Private philanthropy

**Preclinical & CMC:** Foundations, Private philanthropy, Corporate “donations”

**Clinical PI/IIa:** Patient advocacy foundations, Private philanthropy, Research hospitals

**PII & Registration:** Patient advocacy foundations, Private philanthropy, Research hospitals & investors?
SO HOW IS THE EXPERIMENT GOING?

• Started lead series development program December 2017
  • Ontario Institute of Cancer Research (Med Chem, project management)
  • Charles River Laboratories (Med Chem, Assays)
  • Reaction Biology Corp (Assays)
  • GL Chemtec (Chemistry)
  • Structural Genomics Consortium (Structural biology, assays)
    • University of Toronto
    • Oxford University
    • University of North Carolina
  • ICR UK (Med chem, *in vivo* PD model)
  • Sant Joan de Deu (*in vivo* PD model)
  • Tufts University (assays)
  • University of Pennsylvania (*in vivo* PD model)
  • The Brain Tumour Charity
  • Children’s National (*in vivo* PK)
SO HOW IS THE EXPERIMENT GOING?

• Over 300 compounds synthesized to date
• 5 compounds meet our initial TPP and moving to robust candidate selection
• Monthly project meetings broadcast and available on YouTube
  • Attracted pharma to discuss ALK2 project they had abandoned
  • Scientific and clinician providing input
• https://m4kpharma.com/blog/
Future Opportunities

• Experiments have to be repeatable

• Many other opportunities which meet the DIPG profile:
  • The disease represents a dire unmet need;
  • The disease does not fit historical pharmaceutical business models; and
  • The initial technology is derived from the expertise of one of our existing, or future, collaboration partners

• Opportunity for M4ND: Meds For Neurological Diseases
CONTACT

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@m4kpharma