Parkinson’s Disease: Gene Therapy as a Novel Therapeutic Strategy

Forecasting Analysis Excerpt
Gene Therapy is a Lucrative Market

- **$11 billion** market in 10 years (~2025)
- **Compound Annual Growth Rate (CAGR): 48.9%**
  - Positive clinical trial testing
  - Ready for launch
- **Gene Therapy is the next chapter of precision medicine**
5 Major Gene Therapies In Clinical Development

- uniQure
  - AAV2 GDNF (Phase I)
- Voyager Therapeutics
  - AAV-hAADC (Phase I)
- Oxford BioMedica
  - ProSavin (Phase 2b)
- AnSes
  - Collategene (Phase III)
- NeuroLogix
  - NLX-P101 (Phase II)
Market Potential:
National & International

Likely Case: 5% Adoption

US Cases:
1,000,000 Patients

Patients in Stage 4,5
50,000

US Market Opportunity:
$5.26B

Worldwide Cases:
8,500,000 Patients

Patients in Stage 4,5
425,000

World Market Opportunity:
$44.7B
**Market Potential:** National & International

**Ideal Case: 15% Adoption**

**US Cases:**
- 1,000,000 Patients
- Patients in Stage 4,5: 150,000

**US Market Opportunity:**
- $15.7B

**Worldwide Cases:**
- 8,500,000 Patients
- Patients in Stage 4,5: 1,275,000

**World Market Opportunity:**
- $134B
Gene Therapy currently manages disease but is expected to cure in future
  ○ Prosavin: “an additional five years before disease progresses further”
● Eliminates recurring medication costs
● Value-based pricing (discussed later)

“Eventually, gene therapy will become a staple of 21st century medicine.” - Jack McCain, *Biotechnology Healthcare* 2005
Market Potential: National & International

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US Cases: 1,000,000 Patients

Patients in Stage 4,5 50,000

US Market Opportunity: $5.26B

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World Market Opportunity: $44.7B
Market Potential: National & International

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World Market Opportunity: $134B
Future trends indicate strong market potential.

- PD is a unique untapped patient pool
- Strong sector growth
- Large pool of investors willing to fund R&D and trial process
- Industry largely unaffected by economies of scale
- Unsaturated market

<table>
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<tr>
<th>Pharmaceutical Margins</th>
<th>Biotechnology Margins</th>
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<td>~35%</td>
<td>~45%</td>
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Pricing strategies will focus on value based compensation.

- Pricing is not based on cost of development but on value of drug
- High ticket drug prices face opposition from insurers
- Alternative annuity based payment

\[ P(x) = 25,000 + 4,000x \]

*Price = Fixed Gene Therapy Cost + Yearly Annuity*
Total Revenue will compensate the company over time.

Few Things to Consider

- Assuming that 5% of current Parkinson’s Disease Patients enroll in therapy in a 3 year period
- Assuming that only .5% of new Parkinson’s patients undergo therapy
- Yearly annuity not adjusted for inflation
- Very conservative model

\[
f(x) = \sum_{t=0}^{n} \frac{($25,000 \times \text{New Patients}) + ($4,000 \times \text{Ongoing Patients})}{(1.06)^n}
\]
Competition and generics are not immediate concerns.

- Nearly 3,000 patents have been awarded for genes in the US
- A patent ensures exclusivity for 20 years during which a drug would be fully exclusive
- Annuity pricing plan extends the profitability of therapy
Sales and marketing will guide profits.

- Capturing consumer loyalty for drug is necessary.
- Annuity pricing is appealing to insurers because of smaller upfront costs and minimal risk.
- Total marketing costs minimal due to modified oligopoly.

“Nearly 90% of physicians were very or somewhat satisfied with the information received from sales representatives.”
Market Analysis:
Developmental PD Gene Therapies

5 Major Gene Therapies In Clinical Development

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- Heavy emphasis on R&D
- High Investment - with promising drugs and drugs in the pipeline
The breakdown of Parkinson’s into subtypes presents the opportunity for targeted therapies.
The biggest issue for Parkinson’s gene therapy is the selective Blood Brain Barrier.
Gene therapy is a profitable market with high financial potential and VC interest.
There are new and competitive pricing mechanisms which can offer higher returns and better pricing protection.
Preliminary models suggest that even with competitors profits offer fair compensation for drug development.
The five major gene therapies have a good potential for market transition.