Nanomedicine: State of the Market

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Lux Research profile

- Helps clients capitalize on science-driven innovation
- Clients on five continents, primarily large corporations
- Delivers value via weekly intelligence reports, quarterly State of the Market reports, and analyst inquiry
- Subscription-based services in biosciences, energy storage, solar, water and nanomaterials
- Primary research methodology: Focus on proprietary interviews and site visits
  - > 1,400 interviews last year
  - Site visits in 18 countries
- Source proprietary ideas from Lux Research Network of execs + scientists
- Diverse, 40-person team; Ph.D scientists to market researchers

George W. Bush, U.S. President
Josh Wolfe, Director, Lux Research

21st Century R&D Act signing in Oval Office

LR Chairman Peter Hebert on CNBC

LR President Matthew Nordan testifying before U.S. Congress
Outline

- Market overview on nano-enabled drug delivery
- Technologies, development, and who is developing them
- Sample regenerative technologies in nano-medicine
- Q&A/Discussion
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The purpose of “nano” – enabled delivery is to target delivery of active therapeutic compounds.
Delivery is a span of control and targeting

**Untargeted delivery**
- Actives affect unintended tissues
- High cost for large amount of active molecule
- Actives degraded or excreted before reaching target
- Too little of the needed dose reaches the target

**Targeted delivery**
- Lower cost for smaller amount of active molecule and targeting system
- Side effects mitigated or eliminated
- Less active released into the environment
- The right therapeutic dose reaches the target

△ Active ingredient
Nano-enabled targeting system

Source: Lux Research
Untargeted delivery

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Targeted delivery

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- The right therapeutic dose reaches the target
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Targeted delivery:
Technologies for directing the effect of active ingredients to an intended organ, cell type, environment, or other condition (delivery) (targeted)
The market for targeted “nano-enabled” delivery is booming

- 2010 market size: $6.8 billion
- 2015 market size: $21.1 billion
- Other applications for nano-enabled delivery include cosmeceuticals, nutraceuticals, agriculture, and food
- Drug delivery claims 97% of the total targeted delivery market today; 90% by 2015
Scientific publishing and patenting activity are at a fever pitch

Number of published journal articles

Number of issued patents and patent applications

Source: Lux Research
But the space is highly fragmented

Companies with highest number of targeted delivery patents

- Pfizer
- GlaxoSmithKline
- Merck & Co.
- Wyeth
- Procter &
- Bristol-Myers...
- Genentech
- Allergan
- Novartis
- Medtronic
- Eli Lilly and Co.
- Boston Scientific
- Novo Nordisk
- Abbott...
- Amgen
- Novartis Pharma
- Alza
- 3M
- Schering
- Pharmacia
- Schering

Breakdown of targeted delivery developers by type and by 2008 revenue

- 153 Public companies
  - >$1B: 23
  - $500M-1B: 9
  - $100M-500M: 18
  - $50M-100M: 14
  - <$50M: 89

- 220 Private companies

Source: Lux Research
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Identify the engineerable property that gives the type and degree of control needed

- Encapsulation of active
- Particle porosity
- Biochemical affinity
- Environmental activation
- External activation

- Particle size
- Chemicophysical environment
- Particle shape
- Presentation of active
- External direction and accumulation

Increasing control over active ingredient in:
- Time
- Space
- Dose
- Activity
What nanotechnologies enable control of delivery?

- Nanoparticulate reformulations/depot systems
- Nano-encapsulation
- Bio/chemical targeting
- Advanced materials/other
- Drug/device combinations
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Nano-encapsulation

- **Purpose:** protects actives from the environment, and vice versa; improve solubility in vivo
- **Technologies:** micelles, liposomes, emulsions, other amphiphilic molecules or nano-polymers used to protect against degradation
- **Market drivers:** interest from “big pharma”
- **Barriers:** crowded space, but few can legitimately demonstrate efficacy and stability
- **Degree of targeting:** low
Lux Innovation Grid: Nano-encapsulation

- **Technical Value**
  - High-potential
  - Long-shot
  - Dominant
  - Undistinguished

- **Business Execution**
  - Technical Value
  - Business Execution
  - Lux Take
  - Maturity

- **Companies**
  - Lux
  - Innovation Grid
  - Nano-encapsulation
  - NutraLease
  - Novosom
  - PharmaSol
  - NanoCarrier
  - Salvona Technologies
  - Endocyte
  - Aquanova
  - OctoPlus
  - Vive Nano
  - Dermazone Solutions

- **Labeled Points**
  - Vivo
  - NanoCarrier
  - PharmaSol
  - Aquanova

- **Legend**
  - 5.0: Strong Positive
  - 4.0 - 4.9: Positive
  - 3.0 - 3.9: Wait and See
  - 2.0 - 2.9: Caution
  - 1.0 - 1.9: Strong Caution
Nanocarrier: drug reformulations using micelle nanoparticles

- 20 nm to 100 nm spheres comprising biocompatible block copolymers of hydrophilic polyethylene glycol (PEG) and hydrophobic polyamino acid
- Several formulations in clinical trials including encapsulated paclitaxel for stomach cancer, cisplatin, and oxaliplatin, all for cancer
- Also working on proteins, siRNA, and pH or conjugation-based release
- Has shown mild, but clinically significant therapeutic improvements
Bio/chemical targeting

- **Purpose:** direct a payload to a specific location or cell type in vivo
- **Technologies:** varies widely, from receptor/ligand interactions through functionalized nanoparticles to light-activated compounds
- **Market drivers:** need for more specific delivery to reach intended target
- **Barriers:** formulations need to be tailored for each new product, lengthening development cycles
- **Degree of targeting:** medium
Lux Innovation Grid: Bio/chemical targeting

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Lux Take

Maturity

Lux Take

5.0
Strong Positive

4.0 - 4.9
Positive

3.0 - 3.9
Wait and See

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Caution

1.0 - 1.9
Strong Caution

Technical Value

Business Execution

Dominant

High-potential

Long-shot

Armagen Technologies

Frontier Pharmaceutical

Luna nanoWorks

Indiana Nanotech

Dendritech

TransDental Technologies

Potentia Pharmaceuticals

PCI Biotech

Nanovector

Undistinguished

5.0

4.0 - 4.9

Strong Caution

Caution

Wait and See

Positive

3.0 - 3.9

2.0 - 2.9

1.0 - 1.9

Strong Caution
Halozyme: Engineered enzymes for delivery of drugs through the extracellular matrix

- Uniquely enabling technology for delivery of injectible drugs
- Strong partnerships with Roche and Baxter, including more than $60 million in up-front and equity payments and more than $600 million in potential milestones
- Proving efficacy in clinical trials including delivery of fluids, biologics, and small molecules; proprietary coformulations with insulin, chemotherapeutics, and bisphosphonates
- Broad applicability in cancer, diabetes, osteoporosis, and cosmetic/aesthetic medicine
INFUSE-LR Phase IIIb Study: Increased Flow Rates for Subcutaneous Infusion

Randomized double blind placebo-controlled Phase IIIb study of subcutaneous hydration of lactated Ringer's (LR) +/- HYLENE in 54 volunteer subjects

End-Infusion (400 mL)

End-Infusion (400 mL)

HYLENE
Placebo

Side-by-Side Simultaneous Sub-Q Infusions
Drug/device combinations

- **Purpose:** enable fine control over the release of active
- **Technologies:** varies widely from injected reservoir systems to iron-oxide nanoparticles injected for thermal ablation of tumors to metal nanoparticles as imaging agents
- **Market drivers:** create less invasive long-term therapies
- **Barriers:** extremely scattered innovation; large players racing for first mover’s advantage on many indications
- **Degree of targeting:** high
Lux Innovation Grid: Drug/device combinations

- **Lux Take**
  - Maturity
    - Strong Positive: 5.0
    - Positive: 4.0 - 4.9
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    - Strong Caution: 1.0 - 1.9

- **Technical Value**
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- **Business Execution**
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- Companies:
  - Isis Biopolymer
  - pSivida
  - MagForce Nanotechnologies
  - NanoBioMagnetics
  - Nanocyte
  - Cerco Medical
  - Ichor
  - Altea Therapeutics
  - Ichor
MagForce Nanotechnologies: injected iron oxide nanoparticles for tumor ablation

- After careful imaging of glioblastoma, inject iron oxide nanoparticles into tumor
- Apply external magnetic fields to heat up nanoparticles (but not surrounding tissue)
- Nanoparticles reach temperatures of 41 °C to 70 °C and kill cancerous cells
- Particles are injected only once and remain in place for duration of treatment
- Recently completed Phase II clinical trials for advanced glioblastomas

MagForce’s iron oxide nanoparticles are 10-15 nm in diameter

The physician can determine the quantity and placement of nanoparticles before surgical intervention.
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Nano-enabled regenerative medicine

- **Technologies:** tissue regeneration scaffolds, structural implant materials, bone repair, bioresorbable materials, some implantable devices (sensory aids, retina implants etc.), surgical aids, operating tools, and smart instruments

- **Market drivers:** “smart biomaterials” currently a $47 billion dollar opportunity (including spinal repair, active wound care, bone fillers, and orthopedic biomaterials), only to increase with aging baby-boom generation

- **Barriers:** several applications (especially spine-related injury) are very early stage; innovation around implantable devices is incremental – rarely disruptive
Nanotope: peptide-based nanostructured extracellular matrices

- Hydrophilic bioactive “head”: short peptide sequence that exerts an influence of interest on target tissues
- Can be altered to elicit different regeneration across many cell types
- Resorbable scaffold dissolves completely in vivo in about 6 months
- Targeting neuronal, vascular, bone, myocardial, and cartilage tissues
- “Supramolecular Design of Self-assembling Nanofibers for Cartilage Regeneration” (February, 2010)
Summary

- Nano-enabled drug delivery is a *booming market*, and scientific activity is very high.
- Nano-enabled delivery technologies are evolving rapidly.
- Field of developers is fragmented though industry is >20 years old.
- Nano-encapsulation is crowded with *undifferentiated players*.
- Bio/chemical targeting is also crowded, though *technical barriers* prevent stunning successes.
- Drug/device combination space is relatively sparse, with several companies showing promise.
- Nano-enabled regenerative medicine is still *early stage*.
**Conclusions/recommendations**

- Developers working on "platform" technologies open opportunities for collaboration across traditional technical and industry lines.
- Emerging targeted delivery technologies should be evaluated on:
  - Technical merits such as effective dose
  - Business execution and company maturity
  - Points and degree of control over the active molecule
- Innovation is not enough: developers should be prepared to look outside medicine's traditional boundaries for collaborators to hasten commercialization.
Thank you / Q&A

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