

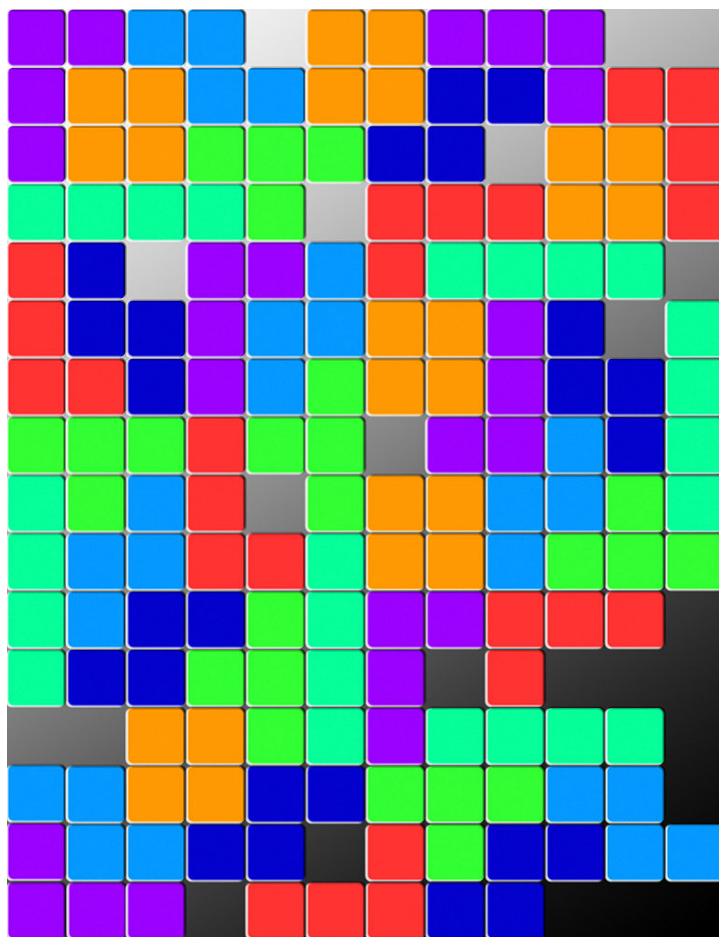


Biotech Patent Pools: CRISPR-Cas9

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BIO International 2017

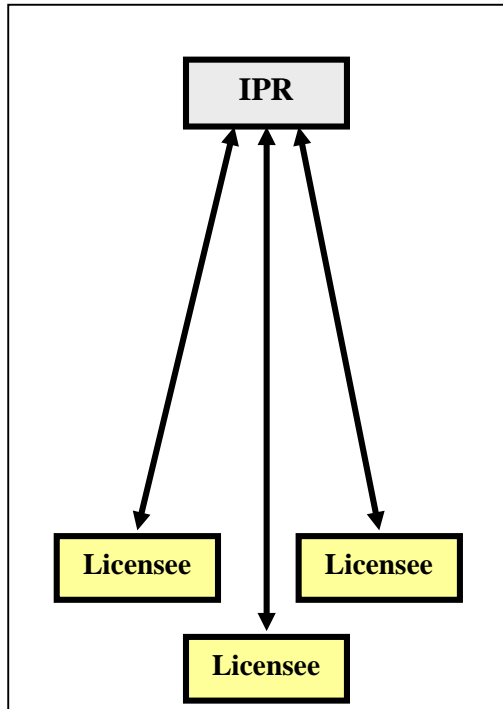
Innovation Relies on Patent Protection



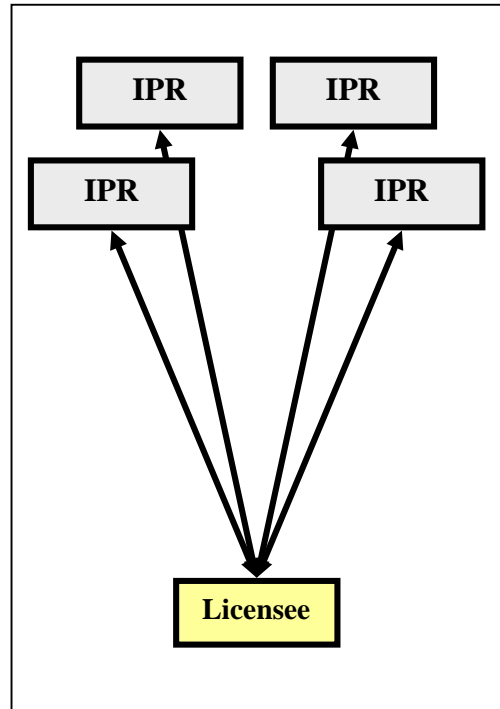
- Foundational patents followed by improvements, specific applications, related technologies held by many different parties
- Can there be licensing efficiency in a crowded, often uncertain and confusing space?

MPEG LA[®] Licensing Model

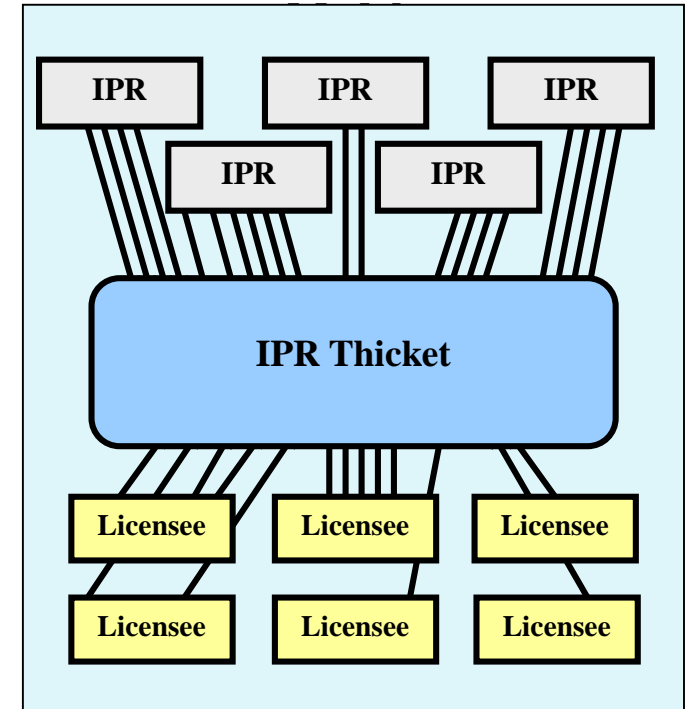
“One-to-Many”



“Many-to-One”



MPEG LA[®]
“Many-to-Many” Licensing



MPEG LA pioneered the modern-day patent pool

MPEG LA[®] Licensing Model

- Starting with the MPEG-2 digital video standard, MPEG LA patent pools have enabled efficient worldwide access to important technologies helping to produce the most widely used standards in consumer electronics history
- The solution has become the template. MPEG LA operates licensing programs consisting of more than 14,000 patents in 84 countries with some 230 patent holders and more than 6,000 licensees.



Molecular Diagnostics

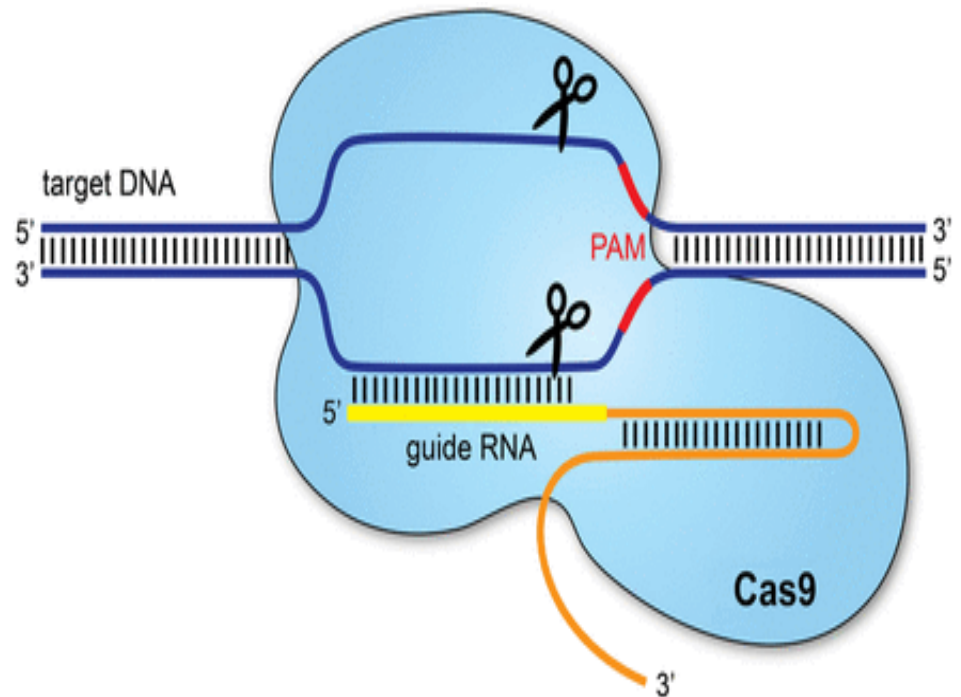
- MPEG LA developed a molecular diagnostics licensing program as a solution to patent access concerns with multiplex test panels and other products
- Nine institutions joined including Johns Hopkins, Ludwig Cancer Research, Memorial Sloan Kettering, NIH, Partners Healthcare, Stanford, University of Pennsylvania, UCSF, WARF



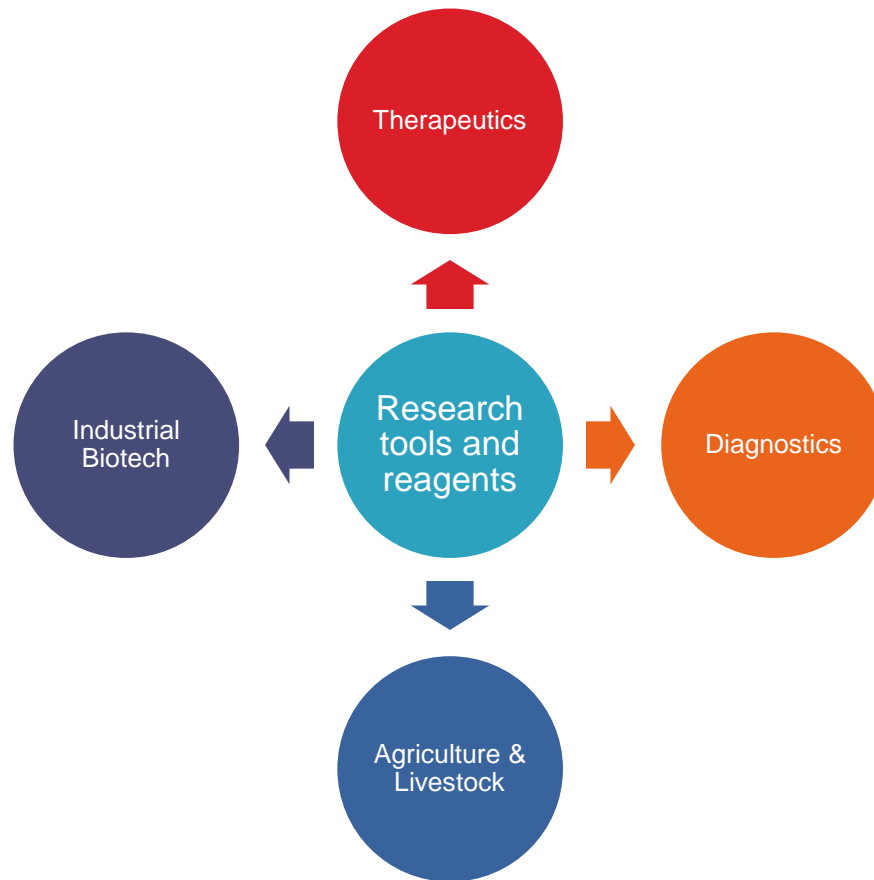
CRISPR-Cas9 System

Fundamental Elements

- **Guide RNA** – single or dual, provided there is guide sequence and trans-activating portion capable of facilitating formation of a complex with the Cas9 protein
- **Cas9 Protein** – wild type or modified, provided it retains the ability to form a complex with the Guide RNA, thereby targeting the Cas9 protein to a target DNA sequence



CRISPR Markets



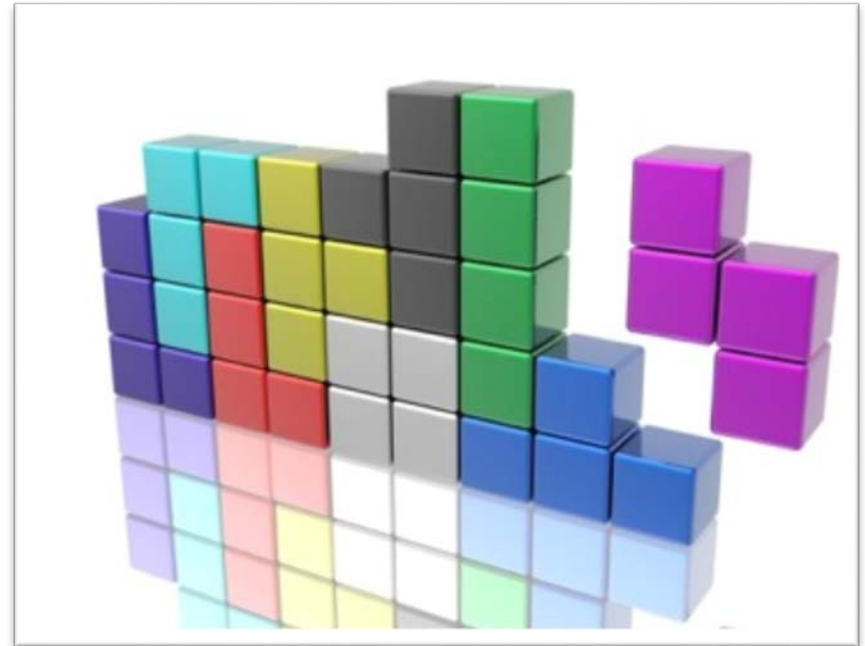
Patent Assets Snapshot

- **University of California/Charpentier**
 - CRISPR-Cas9 systems featuring single guide RNA for use in any environment
- **The Broad/MIT/Harvard**
 - CRISPR-Cas9 systems for use in eukaryotes
- **Vilnius University**
 - CRISPR-Cas9 systems featuring recombinant Cas9-guide RNA complex (RNP complex)
- **ToolGen Inc.**
 - CRISPR-Cas9 systems for use in eukaryotic and mammalian cells
- **MilliporeSigma**
 - Methods for integrating a donor sequence in a eukaryotic chromosome using CRISPR-Cas9 systems

CRISPR Patent Landscape is Growing

IPStudies of Switzerland has classified hundreds of patent families directed to CRISPR-Cas (mainly Cas9)

- Systems
- Components
- Modified cells/organisms
- Methods
- Applications



CRISPR-Cas9 Pool

A Solution Whose Time Has Come

- Vast potential to improve quality of life
- Increasing volume of patents held by multiple entities covering complementary subject matter
- Worldwide mass market
- Business risks and uncertainty threaten robust development
 - Interferences, oppositions, litigations
 - Freedom to operate will not be possible without multiple licenses
 - Even if multiple licenses were possible, stacking royalties, multiple reporting and diligence obligations will be too burdensome
- Voluntary pool in which stakeholders decide CRISPR's destiny in concert with the market is preferable to solutions imposed from on-high (e.g., compulsory licensing, march-in, regulation)
- Pool balances access by many users with return on investment for innovators – while speeding market development and adoption



CRISPR-Cas9 Joint Licensing Platform

Announced December 6, 2016

**MPEG LA to Help Solve the CRISPR Puzzle by
Making the Pieces Easily Accessible to a
Multi-User Market**





Call for Patents

Announced 25 April 2017

Patent holders are invited to submit patents in order to participate on the ground floor of creating the CRISPR-Cas9 Joint Licensing Platform

<http://www.mpegla.com/main/pid/CRISPR/default.aspx>

QUESTIONS?

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