



## Building a Leading Antiviral Franchise



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# Safe Harbor Statement

This presentation includes forward-looking statements about our business, including without limitation statements regarding drug discovery, research and clinical development, regulatory approval processes and market opportunities. These forward-looking statements are subject to important risks and uncertainties that may cause actual events or results to differ materially from our current expectations for a number of important reasons, including those detailed in our publicly-available filings with the Securities and Exchange Commission. In particular, our expectations could be affected by, among other things, unexpected regulatory actions or delays, including with respect to FDA review of IDX184 and IDX19368 or government regulation generally. There can be no guarantee that development of any of our drug candidates described herein will succeed or that any new products will obtain necessary regulatory approvals required for commercialization or otherwise be brought to market. All forward- looking statements speak only as of the date of this presentation and, except as required by law, we undertake no obligation to update such statements.

# HCV Pipeline Overview

Product Candidate	Indication	Preclinical	Phase I	Phase IIa	Phase IIb	Phase III	Market	
<i>INDEPENDENT DEVELOPMENT PROGRAMS</i>								
IDX184* (Nucleotide Inhibitor)	HCV	▶						
IDX719 (NS5A Inhibitor)	HCV	▶						
IDX19368** (Next-Generation Nucleotide Inhibitor)	HCV	▶						
Uridine nucleotide analog	HCV	▶						
Additional Nucleotide Inhibitors	HCV	▶						

\*In August 2012, IDX184 was placed on partial clinical hold by the FDA.

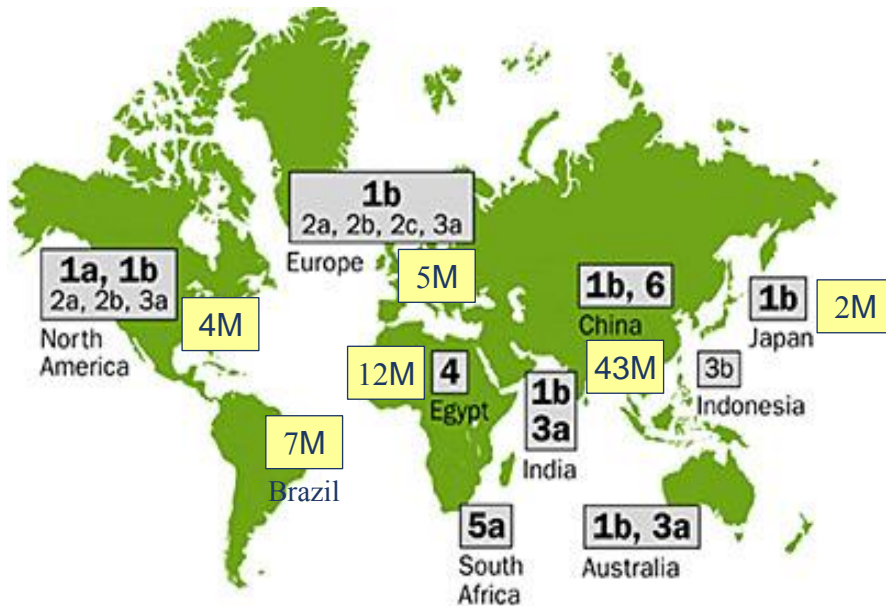
\*\*In August 2012, IDX19368 was placed on clinical hold by the FDA.

# Idenix Pharmaceuticals Highlights

- Focused on development of next-generation oral antiviral combination treatments for hepatitis C virus (HCV) infection
- IDX719, an HCV NS5A inhibitor, completed 3-day proof-of-concept study
  - Favorable safety and potent pan-genotypic activity in HCV-infected patients
  - All-oral phase II combination trials expected to initiate 1H 2013
- HCV nucleotide inhibitor program
  - Next-generation uridine nucleotide analog in IND-enabling studies; filing 1H 2013
  - IDX184 (phase IIb) and IDX19368 (IND filed 3Q12) placed on clinical hold by FDA in August 2012 due to cardiac-related SAEs with BMS-094 (formerly INX-189)
  - Robust discovery efforts ongoing
- Restructured agreement with Novartis enhances Idenix's strategic flexibility to develop an optimal DAA portfolio of both internally and externally derived assets
- 2013 will bring multiple paths to evaluate all-oral combination HCV regimens

# HCV is a Viral Disease that Affects the Liver, with ~170M Infected Individuals Worldwide

## Epidemiology and distribution of HCV genotypes in the world

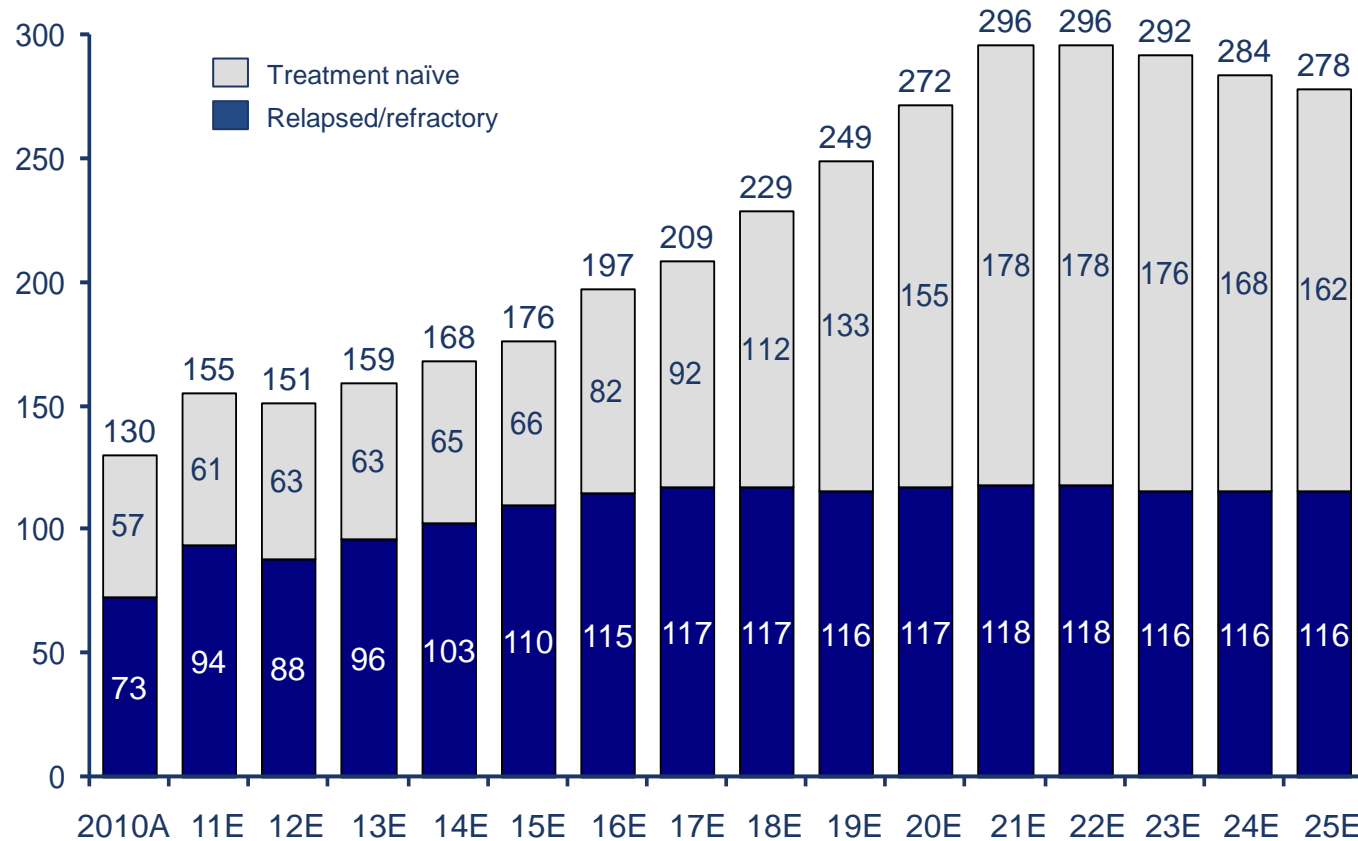


- Of the 170M HCV-infected, only ~11M reside in developed countries
- 6 different HCV genotypes WW with genotypes 1, 2 and 3 comprising the majority of patients
- Current DAA combinations primarily address genotype 1
- Pan-genotypic treatments can be used widely in both developed and emerging countries
  - Roughly half of WW HCV-infected are non-GT1

Source: WHO, Industry analyst reports, BioCentury, L.E.K. interviews and analysis

# CDC Guidelines Driving Increased Diagnosis will Result in More Sustainable Patient Population Needing Treatment

**Treated patients in the U.S., EU, and Japan (2010-25E)**  
Thousands

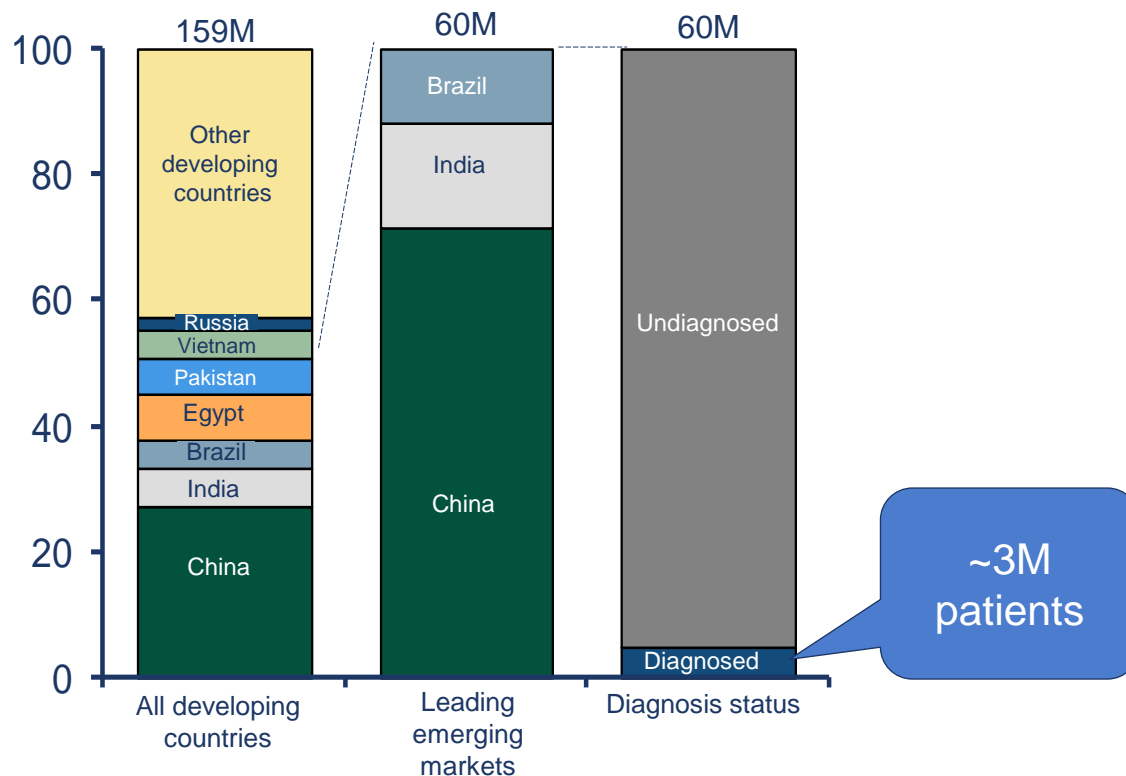


Source: Industry analyst reports, L.E.K. interviews and analysis

# Developing Markets also Represent a Significant Opportunity as Prevalence is High but Under-Diagnosed

## HCV prevalence in developing countries (2011)

Percent of patients



Today, emerging markets represent an opportunity of ~3M diagnosed patients; however, this is likely to grow dramatically as diagnosis rates are expected to increase significantly

Source: Industry analyst reports, Clinical Liver Disease, HCV Advocate, L.E.K. interviews and analysis

# HCV Competitive Landscape

## *Significant Unmet Needs Beyond First-to-Market All-Oral Regimens*

### Current DAA Combinations

**Potent**  
**Safe**  
**Convenient**  
**High barrier to resistance**  
**Genotype 1 focused**  
**Treatment-naive**

### Future DAA Combination Opportunities

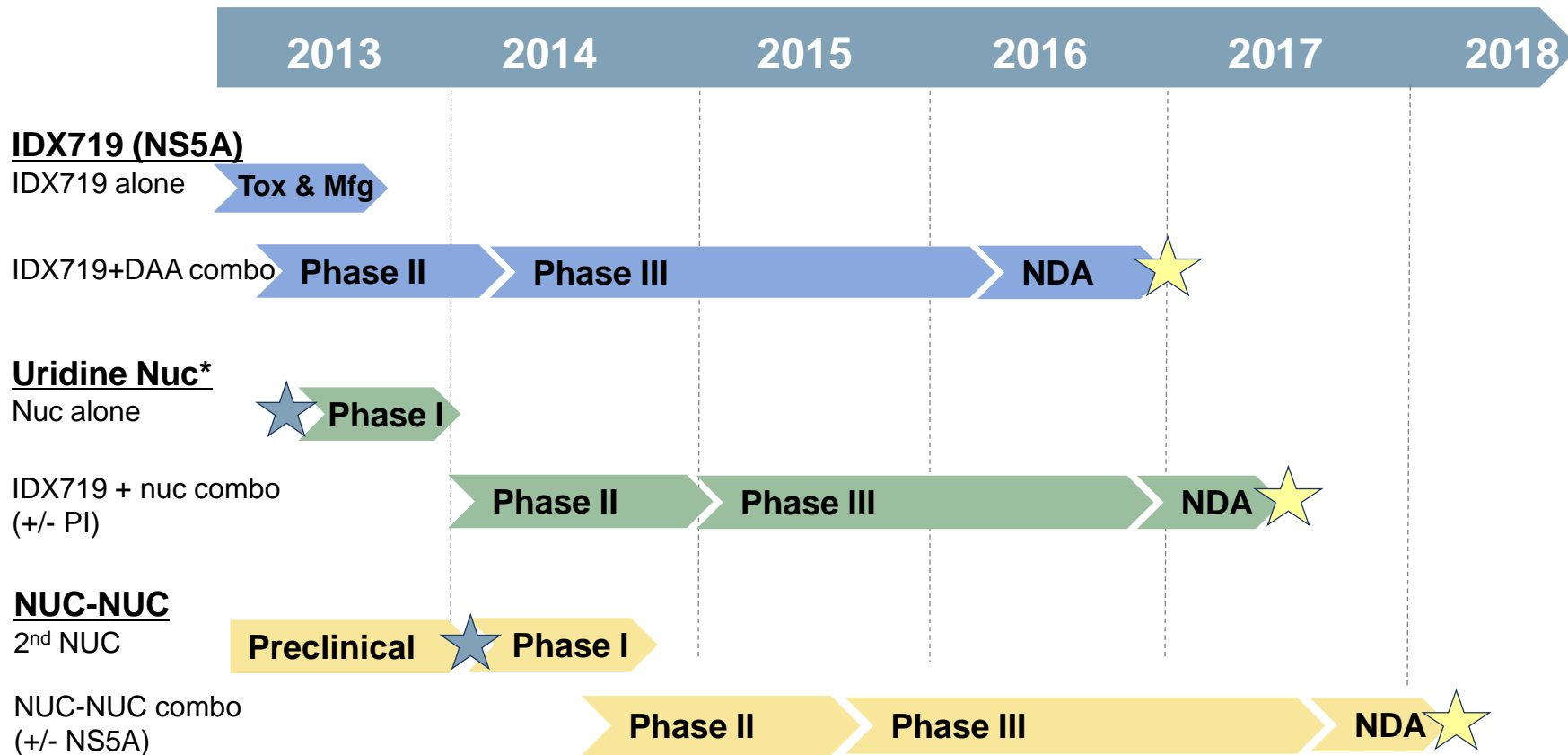
**Pan-genotypic regimen**  
**Null responders**  
**Advanced fibrotic patients**  
**Pre-transplant patients**  
**Cirrhotic patients**  
**Protease inhibitor failures**  
**HIV co-infected patients**  
**Emerging markets**



# Idenix HCV Clinical Development Strategy

## Multiple Paths Forward for DAA Combinations

HCV Strategy



\*IND-enabling studies for follow-on nucs underway

★ IND ★ Regulatory approval

# IDX184 and IDX19368 Update

- In August 2012, FDA placed IDX184 on partial clinical hold and IDX19368 on clinical hold due to cardiac SAEs seen with BMS-094 (formerly INX-189)
- The three compounds share the same active metabolite (2'-methyl G); however, we believe there are significant differences between IDX184 and IDX19368 compared to BMS-094
- No evidence to date of clinical cardiotoxicity in patients dosed with IDX184 with PegIFN/RBV beyond that seen with PegIFN/RBV alone; no patients exposed to IDX19368
- In December, IDX184 response package submitted to FDA
- In January, FDA communicated that they will require additional time to review the submission
- Anticipate FDA response in 1Q 2013

# IDX719: Best-in-Class Profile Among HCV NS5A Inhibitors

- Strong preclinical profile
  - Potent, pan-genotypic activity *in vitro* (2-24 pM)
  - Clean safety
  - Low potential for drug-drug interactions
- Three-day proof-of-concept clinical trial in 64 HCV-infected patients demonstrated safety and pan-genotypic activity
- Three-month toxicology and solid-dose formulation work complete to support phase II clinical trials
- 12-week phase II all-oral combination clinical trial expected to begin 1H 2013

# IDX719: Phase I/II Study

## Potent and Pan-genotypic in 3-Day Proof-of-Concept Study

- Well tolerated with no treatment-emergent serious adverse events reported
- Potent antiviral activity across genotypes in HCV-infected patients:

Dose	Mean Maximum Viral Load Reduction				
	GT1a n=23	GT1b n=5	GT2 n=8	GT3 n=8	GT4 n=8
25 mg QD	3.3 log <sub>10</sub>	3.0 log <sub>10</sub>	--	--	--
50 mg QD	3.6 log <sub>10</sub>	4.3 log <sub>10</sub>	--	--	--
50 mg BID	3.2 log <sub>10</sub>	--	2.0 log <sub>10</sub>	3.3 log <sub>10</sub>	3.9 log <sub>10</sub>
100 mg QD	3.5 log <sub>10</sub>	--	2.0 log <sub>10</sub>	3.4 log <sub>10</sub>	3.4 log <sub>10</sub>

# IDX719: First NS5A to Show Pan-genotypic Activity as Monotherapy in the Clinic

	GT1	GT2	GT3	GT4
IDX719	✓	✓	✓	✓
BMS-790052	✓			
GS-5885	✓			
ACH-2928	✓			
PPI-461	✓			
PPI-668	✓		✓	
ACH-3102	✓			
ABT-267	✓			
GSK-805	✓			

Source: Company public disclosures

# Novel Nucleotide Prodrug Discovery Program

- Diverse spectrum of nucleotides
  - Purines and pyrimidines
  - Known prodrugs and novel prodrugs
  - 2' Me sugars and some novel sugars
- Identify promising compounds *in vitro* and in mouse and monkey
  - Level of triphosphate (TP) production, kinetics of metabolism, cytotoxicity, etc
  - Levels of TP in the liver after oral administration *in vivo*
- Additional INDs expected to be filed in 2013
  - Uridine analog is first candidate selected
  - Potential for HCV nuc-nuc strategy; nucs with non-overlapping resistance profiles
- Nuc discovery capability can also be applied to other therapeutic areas
  - Potential uses in non-HCV therapeutic areas
  - External interest in screening library
  - Restructured Novartis agreement allows flexibility to explore other indications

# Idenix Nucleotide Prodrugs: Culmination of Intensive Discovery Effort

## *Raising the Bar for Next-Generation HCV Nucs*

>280 Different  
Nucleoside Analogues

>30 Different  
Prodrug Types

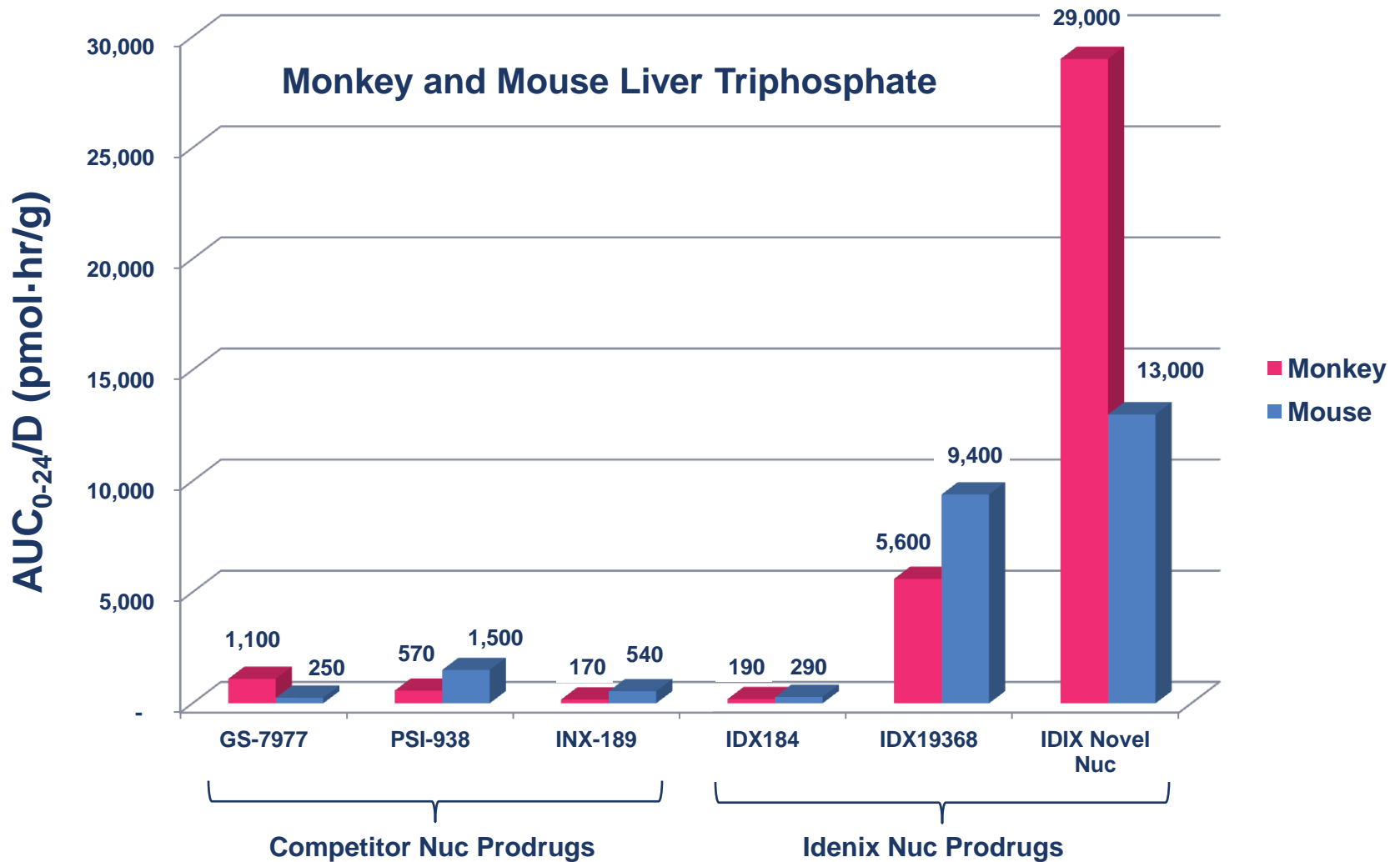
>2100 Nucleotide Prodrugs

>950 Individual Prodrug  
Diastereomers Separated

Liver TP Levels *In Vivo* for:  
>220 prodrugs in mouse  
>15 prodrugs in monkey

**Goals: Improved TP levels *in vivo*, liver-targeting, preclinical safety**

# New Idenix Nucleotide Prodrugs Deliver High Triphosphate Levels



Source: data on file



# Summary of Patent Interference

- February 2012: Interference declared between Idenix patent application (Application 12/131,868) and Gilead issued patent (7,429,572) by the USPTO Board of Patent Appeals and Interferences
- Two phases to Interference
  - First Phase: one party determined to be the Senior Party
  - Second Phase: one party determined to be the First to Invent
- Q1 2013: expected decision in the First Phase of the Interference
- By year-end 2013: expected conclusion to motions and other filings with respect to Second Phase
- By end of Q1 2014: expected decision on Second Phase of the Interference
- Decision of USPTO Board is appealable to federal court

# Financial Highlights

\$ Millions (unaudited)

	Q1 2012	Q2 2012	Q3 2012
Total Revenue	\$35.6	\$1.4	\$32.3
R&D Expense	\$18.6	\$20.5	\$13.5
G&A Expense	\$4.8	\$5.9	\$6.2
Net Income (Loss)	\$11.4	\$(25.4)	\$4.3

\$ Millions

As of December 31, 2012

Cash Balance	\$230.8
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# Anticipated 2013 Milestones

## ■ IDX719 Combination Studies

- Initiate 12-week phase II all-oral combination clinical trials for IDX719
- Initiate all-oral combination study with IDX719 and uridine nucleotide analog

## ■ Nucleotide Inhibitor Program

- Determine next steps for IDX184 and IDX19368 development programs
- Conduct 7-day proof-of-concept study for uridine nucleotide analog
- Complete IND-enabling studies for follow-on nucleotide analogs

## ■ Other

- Decision in First Phase of USPTO patent interference