



TIBSOVO[®] (ivosidenib) FDA Approval

July 20, 2018



Agios Conference Call Participants

Prepared Remarks

Introduction

- KENDRA ADAMS, Sr. Director, Investor Relations

Opening Remarks

- DAVID SCHENKEIN, M.D., Chief Executive Officer

USPI Review

- CHRIS BOWDEN, M.D., Chief Medical Officer

Commercial Launch

- STEVE HOERTER, Chief Commercial Officer

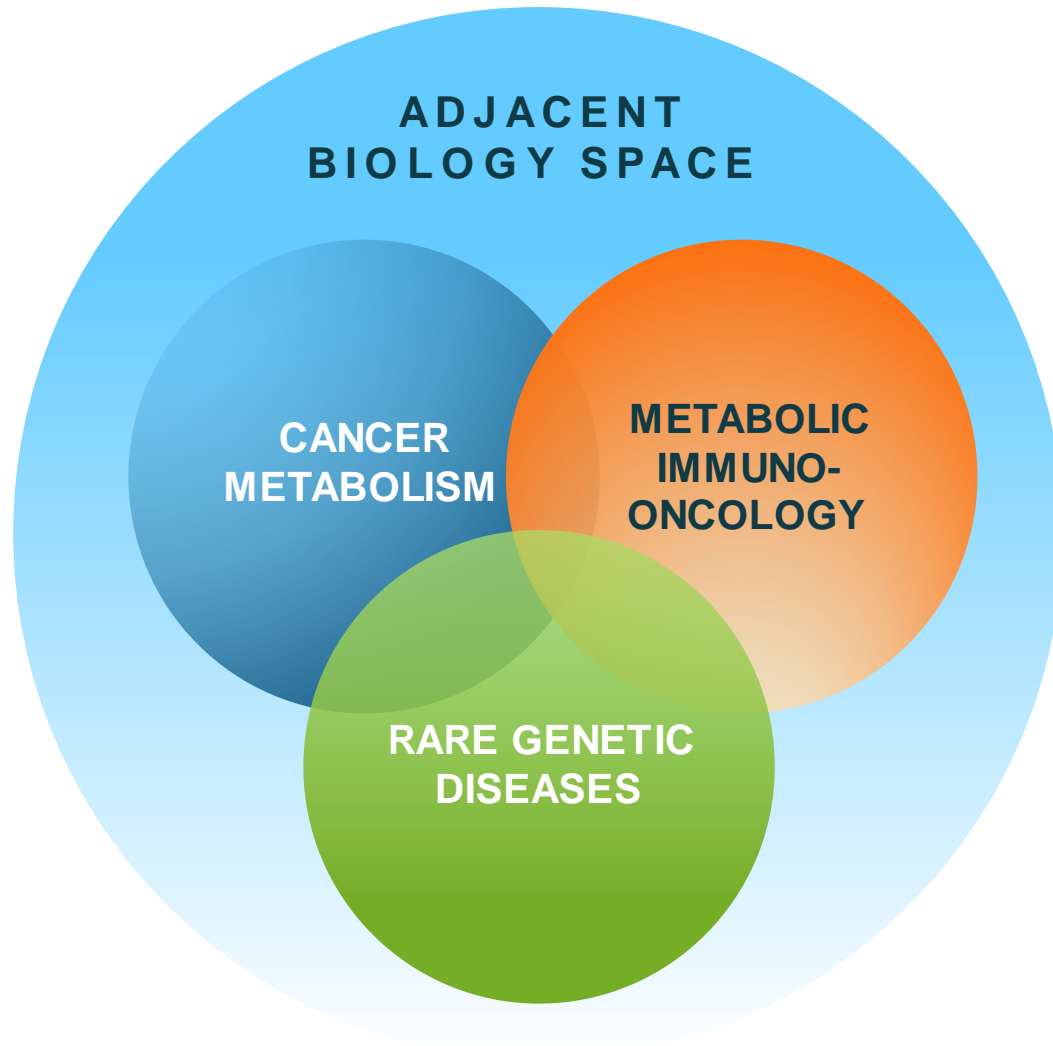


Forward Looking Statements

This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of Agios' products, including TIBSOVO® (ivosidenib), and its strategic plans and focus. The words "estimate," "may," "milestone," "potential," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from Agios' current expectations and beliefs. For example, there can be no guarantee that development of any of Agios' product candidates will successfully continue, or that any positive developments in Agios' business will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this presentation and various remarks we make during this presentation could also be affected by risks and uncertainties relating to a number of other important factors, including: Agios' results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; Agios' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials; unplanned cash requirements and expenditures; competitive factors; Agios' ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates it is developing; Agios' ability to maintain key collaborations, such as its agreements with Celgene and CStone Pharmaceuticals; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in Agios' public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this presentation and various remarks we make during this presentation speak only as of the date hereof, and Agios expressly disclaims any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.



Driven By a Clear Vision and Values



Agios is passionately committed to applying our scientific leadership in the field of cellular metabolism to transform the lives of patients with cancer and rare genetic diseases.



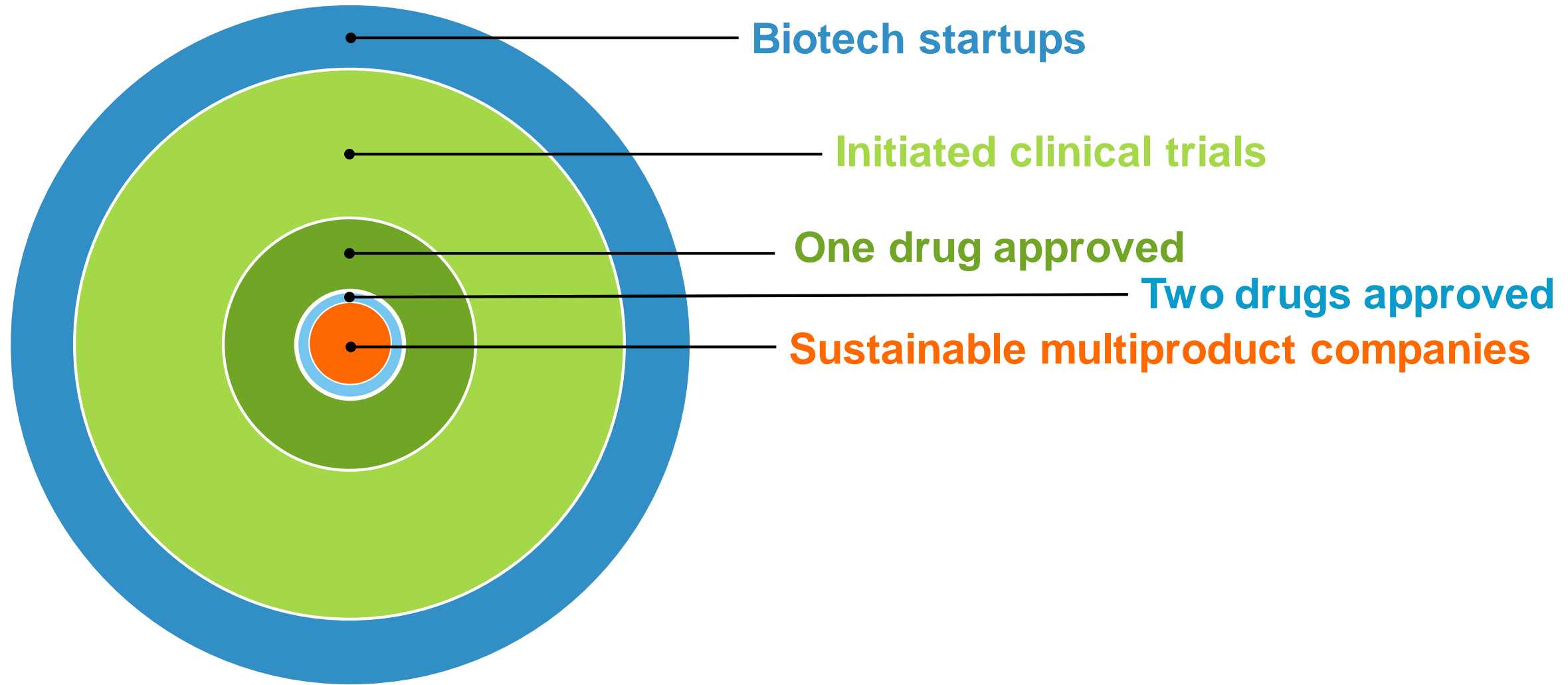
Now Approved in IDH1m Relapsed/Refractory AML



TIBSOVO is indicated for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (AML) with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation as detected by an FDA-approved test.



Building One of the Next Great Pharmaceutical Companies





TIBSOVO[®] U.S. Prescribing Information Review

Chris Bowden, M.D., Chief Medical Officer



TIBSOVO® USPI Highlights*

First-in-class, oral, targeted inhibitor of mutant IDH1 protein

Efficacy Data (n=174)

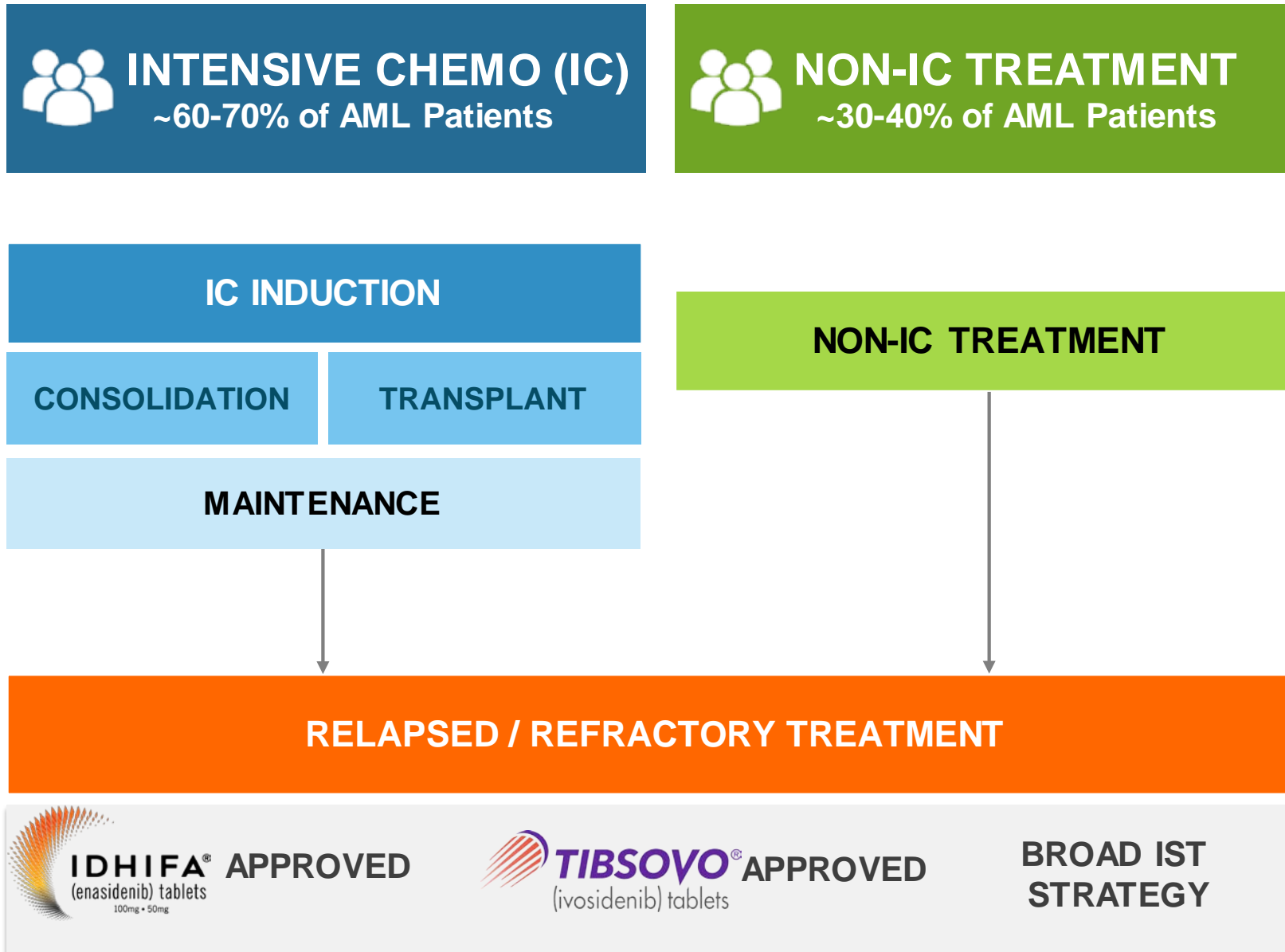
- CR/CRh statistics
 - Rate: 32.8%
 - Median duration: 8.2 months
 - Median time to best response 2.0 months
- Transfusion independence
 - 37.3% of patients became transfusion independent during any 56-day post-baseline period
 - 59.4% of patients independent at baseline remained independent during any 56-day post-baseline period
- 12% of patients went on to stem cell transplant following TIBSOVO® treatment

Safety Data (n=179)

- The TIBSOVO® label contains a boxed warning for differentiation syndrome, which can be fatal if not treated
 - 19% of patients experienced differentiation syndrome (all Grades)
- QTc interval prolongation and Guillain-Barre Syndrome occurred in patients treated with TIBSOVO®
- Monitor drug-drug interactions with TIBSOVO.
- Most frequent serious adverse reactions (≥5%): differentiation syndrome (10%), leukocytosis (10%) and QT prolongation (7%)
- Median duration of exposure: 3.9 months




Clinical Development of IDHm Inhibitors Spans All Treatment Lines to Become Cornerstone of AML Treatment



HOVON 7+3
PHASE 3
PLANNED

BROAD IST
STRATEGY


AGILE study for IDH1 mutation in acute myeloid leukemia
ONGOING

BROAD IST
STRATEGY

Multiple Opportunities Across IDHm Hematologic and Solid Cancers Originating from Agios Research Platform

ACUTE MYELOID LEUKEMIA	CHOLANGIOCARCINOMA	LOW GRADE GLIOMA	OTHER INDICATIONS
<p>IDH2m R/R <i>IDHIFA[®] (enasidenib) Approved</i></p>	<p>IDH1m R/R <i>Ivosidenib Phase 3 (ClarIDHY) Ongoing</i></p>	<p>IDH1m <i>Ivosidenib & AG-881 Perioperative Study Ongoing</i></p>	<p>MYELODYSPLASTIC SYNDROMES</p>
<p>IDH1m R/R <i>TIBSOVO[®] (ivosidenib) Approved</i></p>	<p>IDH1m R/R <i>Ivosidenib Phase 1 Enrollment Complete</i></p>	<p>IDH1m <i>Ivosidenib Phase 1 Enrollment Complete</i></p>	<p>IDHm R/R <i>Ivosidenib Phase 1 Enrollment Complete</i></p>
<p>IDH1m Frontline Non-IC <i>Ivosidenib + Aza Phase 3 (AGILE) Ongoing</i></p>		<p>IDH1m <i>AG-881 Phase 1 Enrollment Complete</i></p>	<p>CHONDROSARCOMAS</p>
<p>IDHm Frontline IC-Eligible <i>Ivo/Ena + 7+3 Phase 3 Q4 2018 Start</i></p>			<p>IDH1m R/R <i>Ivosidenib Phase 1 Enrollment Complete</i></p>
<p>IDHm Frontline Non-IC <i>Ivo/Ena + Aza Phase 1/2 Ongoing</i></p>			
<p>IDHm Frontline IC-Eligible <i>Ivo/Ena + 7+3 Phase 1b Ongoing</i></p>			





TIBSOVO[®] Commercial Launch

Steve Hoerter, Chief Commercial Officer



Strategic Imperatives for the TIBSOVO® Launch

Physicians test
for IDH1m



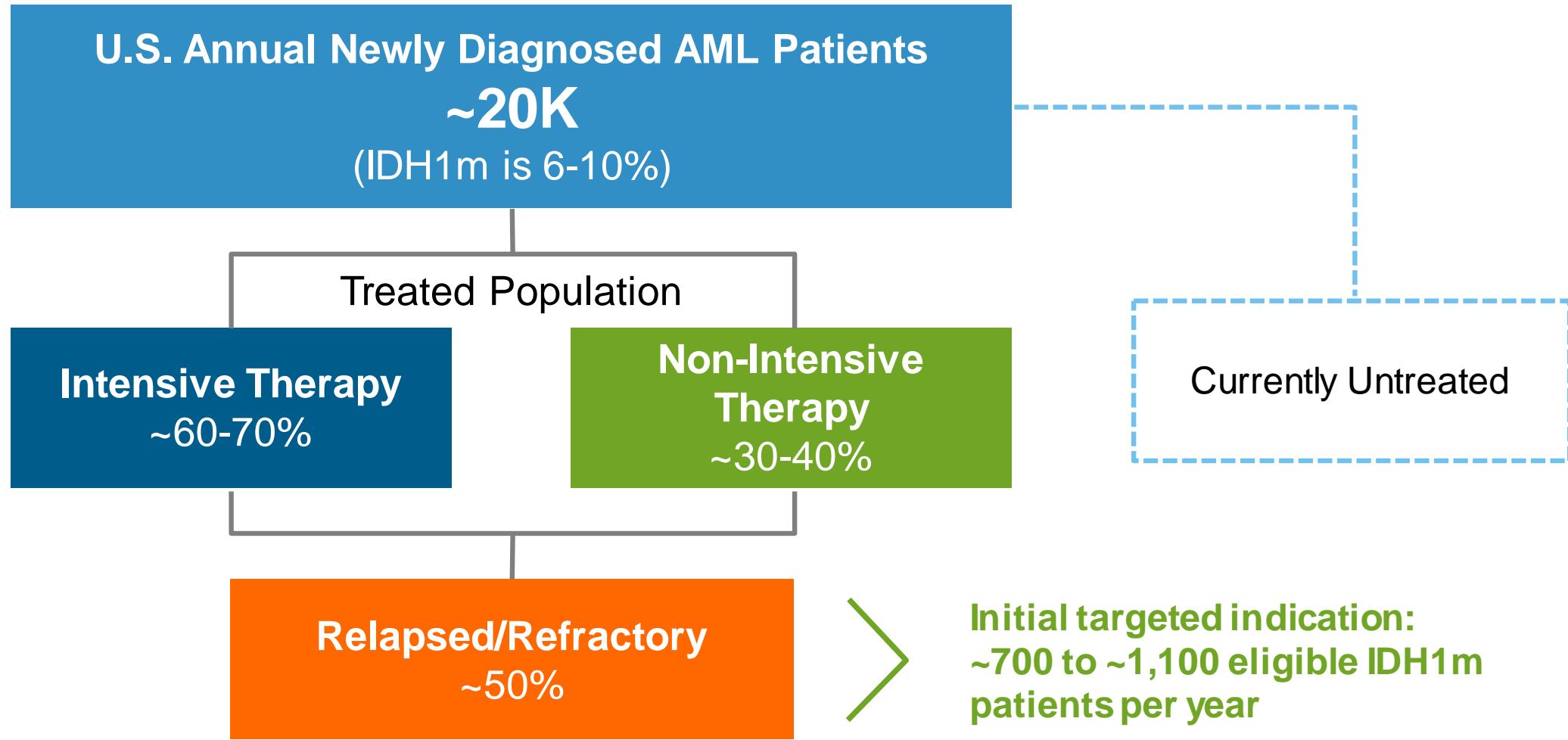
TIBSOVO® is
recognized as
the best option
for IDH1m+
R/R AML



Patients have
access to
TIBSOVO®

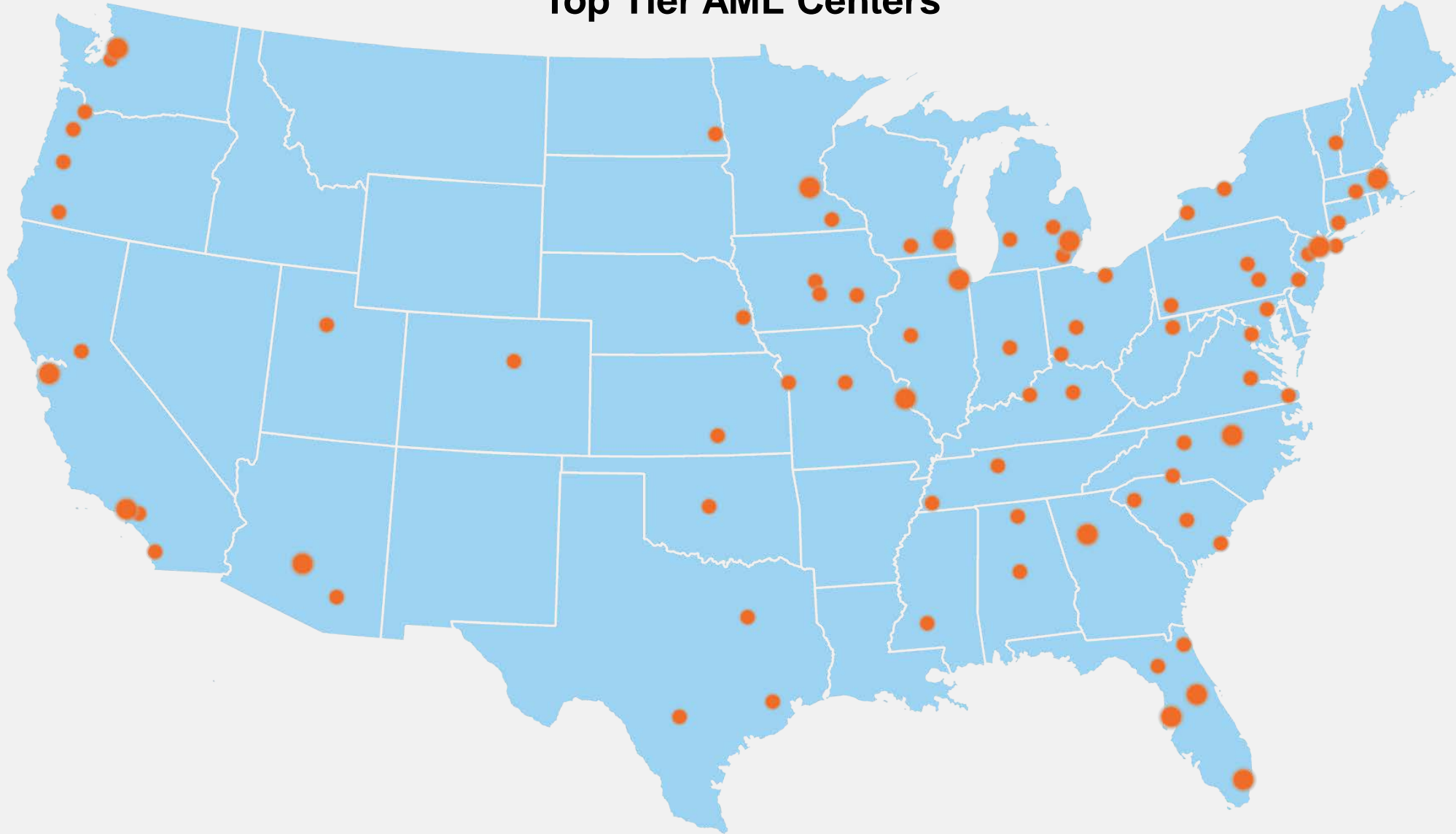


U.S. AML Epidemiology and Treatment Approach – IDH1 Opportunity



Sales Team Deployed to Cover Prescriber Base

Top Tier AML Centers



Our Approach to Patient Access is Multifaceted

Patient Access



Distribution Channel

Limited specialty
pharmacy network

Hospital accounts
serviced via specialty
distributors



Patient Services

Copay card for commercial
patients

Patient Assistance Program

Commercial insurance
coverage interruption

Referrals to independent
third-parties for additional
support



Payer Education

Expert field payer team

Focus on education &
payer resources

Educate on disease
burden

Address payer concerns





[Home](#)

[Financial assistance](#)

[Distribution network](#)

[Enroll now](#)

[Resources](#)



Welcome to myAgios™ Patient Support Services

A program that helps with access and financial assistance



Financial Assistance



Network of Specialty Pharmacies and Distributors



Enrolling in myAgios™ Patient Support Services

Q&A