

# AML: KOL Insight [2017]

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## Abstracts

Will novel targeted therapies change AML treatment pathways?

For decades, chemotherapies have been at the forefront of AML treatment. How is this all set to change within the next few years? Novartis' midostaurin and Seattle Genetics vadastuximab talirine form part of a rich pipeline, but which drugs stand out to key opinion leaders (KOLs)? Are these agents effective enough to displace chemotherapies and lead to durable remissions? Most importantly, will they improve survival in patients that need it the most?

Learn how KOLs see the market evolving, and how they expect developers to differentiate their pipeline therapies in KOL Insight: Acute Myeloid Leukaemia (AML).

Twelve US and European KOLs give their insight on two off-label marketed products and 16 pipeline programmes. KOLs also provide their candid views on the potential for novel chemotherapies and early-stage pipeline programmes.

Take a tour of the report now:

The table of contents

The key business questions answered

The key KOL quotes

See the 18 therapies covered

Find out who the 6 EU & 6 US KOLs are

## Review an extract from the report - 1 drug profile

### Top Takeaways

With an urgent need for effective treatment options in AML, what are key players doing to tackle the deadly disease? How can the unmet needs be addressed with novel pipeline therapies?

Chemotherapies have dominated the treatment algorithm for 40 years, but how is that all set to change within the next 2-3 years? Find out if KOLs believe chemotherapies will continue to play a key part in AML treatment, and how the landscape could evolve.

Four FLT3 inhibitors are in the pipeline; what impact are they likely to have and how can they successfully differentiate themselves? Novartis' midostaurin is leading the race with next generation FLT3 inhibitors hot on its heels. Find out how KOLs will weigh up these new drug options.

How do KOLs feel about other personalised approaches and will they shape treatment? Celgene/Agios' IDH2 inhibitor enasidenib and Roche's idasanutlin are targeting specific pathways in AML, where do KOLs see these niche agents fitting in?

Durable responses are a must, so how does this factor into KOL opinions about novel drugs? Maintaining durable remissions is crucial in AML. Find out which drugs KOLs think will be best suited to maintain patients in remission and prevent relapses.

Is Seattle Genetics's anti-CD33 agent, vadastuximab talirine, poised for success or will it face similar hurdles as Pfizer's Mylotarg? Mylotarg had safety issues but how do KOLs view vadastuximab's potential?

Will novel chemotherapies have a chance to improve upon older agents and if so, what challenges do they face? Novel chemotherapies such as Jazz Pharmaceuticals' Vyxeos and Astex' guadecitabine are hoping to compete with conventional standard-of-care regimens. Find out what KOLs think about this.

What other early-stage pipeline programmes are KOLs particularly excited

about? The early-stage pipeline for AML has diverse mechanisms in Phase II development. AbbVie/Roche's venetoclax has been a game-changer in CLL but does the same hold true for AML?

Combination approaches versus monotherapies; what will succeed in AML? Efficacy, safety and convenience of administration will all play an important role in determining preferred treatments but why are some approaches favoured over others?

## Quotes

"Hopefully, over the next five years, we will first of all see an emergence of widespread availability of these targeted small molecule agents; specifically the FLT3 inhibitors and the IDH1/2 inhibitors, I think they are the most promising compounds." EU Key Opinion Leader

"FLT3 inhibitors, IDH inhibitors, venetoclax; those are very real and I think those will come to the market very quickly. The others are perhaps the monoclonal antibodies, I expect those to make it. I think those are more realistic and the other [drugs] are possibilities but a little further behind." US Key Opinion Leader

## Sample of therapies covered

### Marketed Therapies (off-label)

Nexavar (sorafenib; Bayer/Onyx Pharma)

Mylotarg (Pfizer)

### Pipeline Therapies

midostaurin (Novartis)

quizartinib (Daiichi Sankyo)

gilteritinib (Astellas Pharma)

enasidenib (Celgene/Agios Pharma)

vadastuximab talirine (Seattle Genetics)

Vyxeos (CPX-351; Jazz Pharmaceuticals)

Venclexta/Venclyxta (venetoclax; AbbVie/Roche)

## Sample of KOLs interviewed

### KOLs from North America

Dr Farhad Ravandi, MD, Professor and Chief in the Department of Leukaemia at MD Anderson, Houston, Texas.

Dr Elihu Estey, MD, Professor of Medicine, Division of Hematology, University of Washington School of Medicine, Seattle.

Dr Jorge Cortes, MD, Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, Texas.

### KOLs from Europe

Professor Alan Burnett, MD, PhD, Professor and Head of the Department of Haematology at the University of Wales College of Medicine, Cardiff, UK.

Professor Francesco Lo-Coco, MD, PhD, Full Professor of Haematology and Head of the Laboratory of Integrated Diagnosis of Oncohaematologic Diseases at the Department of Biopathology of the University Tor Vergata of Roma, Italy.

Professor Xavier Thomas, MD, Department of Haematology, Edouard Herriot Hospital, Hospices Civils de Lyon, Lyon, France.

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