

WHAT'S NEW?

...IN MDS, APLASTIC ANEMIA, PNH

MDS

TREATMENTS FOR MDS: THE DARK AGES (BEFORE 2000)



2015: TREATMENT IS AIMED AT THE TYPE OF MDS

LOWER RISK

Erythropoietin

Lenalidomide

ATG

Improve blood counts
Reduce symptoms

HIGHER RISK

Azacytidine

Stem cell transplant

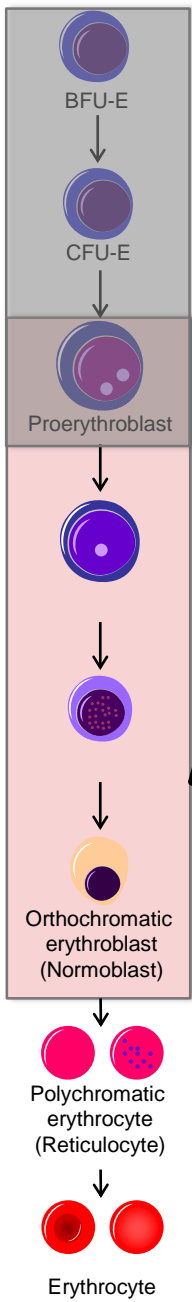
Improve lifespan
Reduce risk of leukemia
Improve blood counts
Reduce symptoms

LOWER-RISK MDS

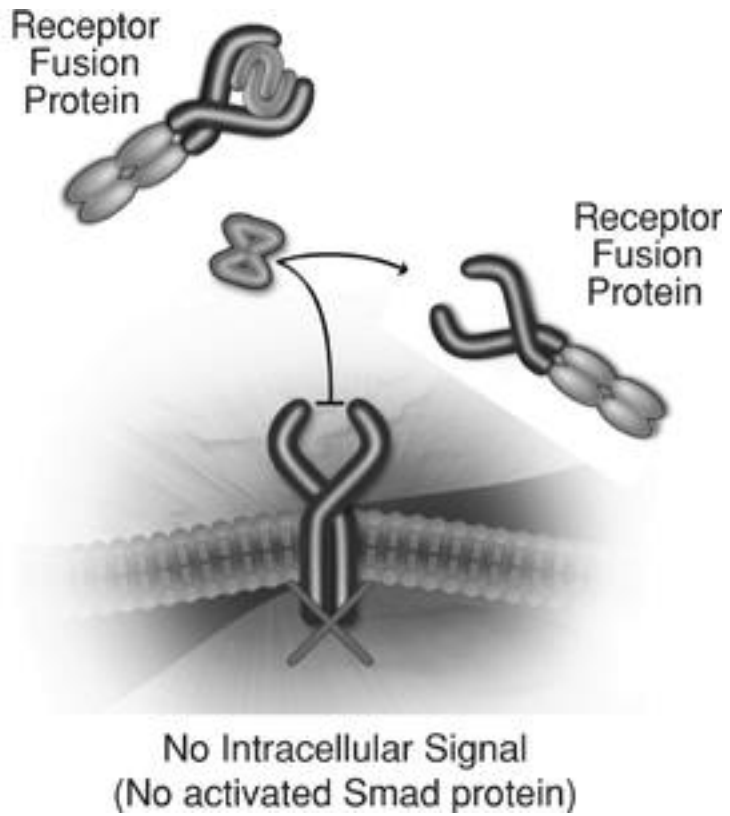
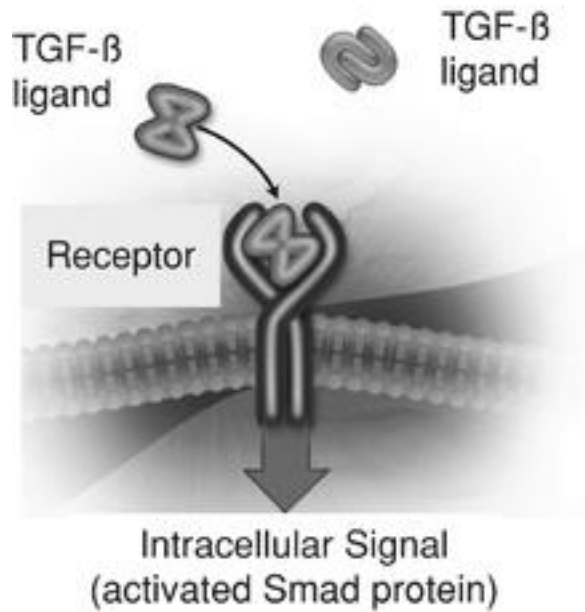
Gaps:

What if Erythropoietin and Lenalidomide fail?

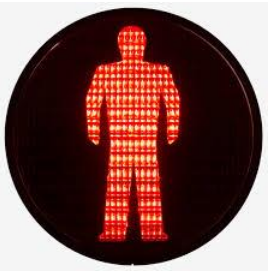
NEW THING: LUSPATERCEPT



Epo works here



TGF- β works here



LOWER-RISK MDS

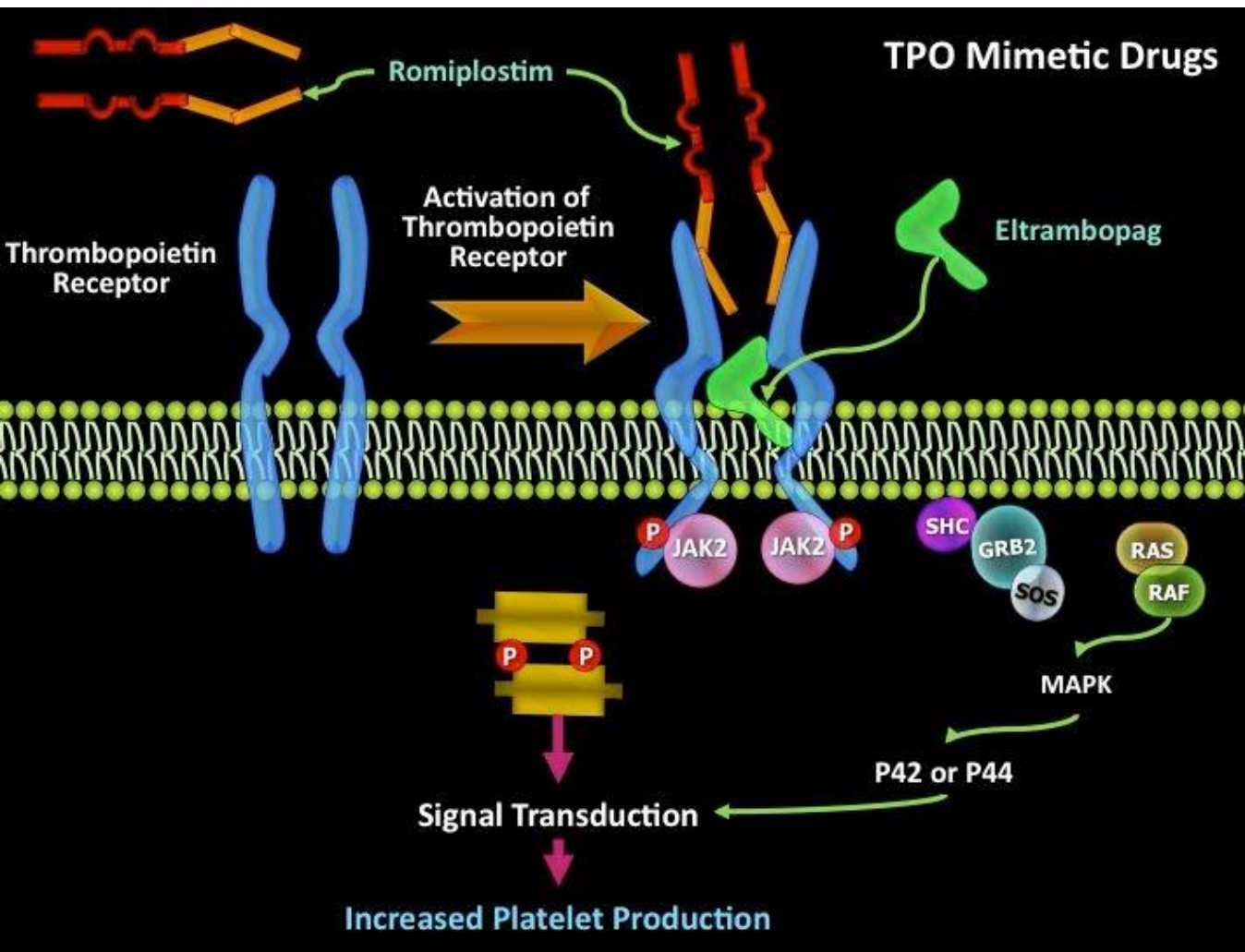
Gaps:

What about symptoms from low platelets?

NEW THING: TPO-MIMETICS

TPO-MIMETICS

ELTROMBOPAG AND ROMIPILOSTIM



LOWER-RISK MDS

What if multiple lineages are down?

We have: Immunosuppressive therapy

WHATS NEW?

Oral azacytidine

Oral decitabine

HIGHER RISK MDS

Treatment is aimed at stopping development of acute leukemia and prolonging life

We have:

Allogeneic stem cell transplantation

Azacytidine

Gaps:

What do we do when Azacytidine stops working?



APLASTIC ANEMIA

APLASTIC ANEMIA

Treatment options:

IMMUNOSUPPRESSIVE THERAPY

ALLOGENEIC STEM CELL TRANSPLANTATION

APLASTIC ANEMIA

Treatment options:

IMMUNOSUPPRESSIVE THERAPY

ALLOGENEIC STEM CELL TRANSPLANTATION

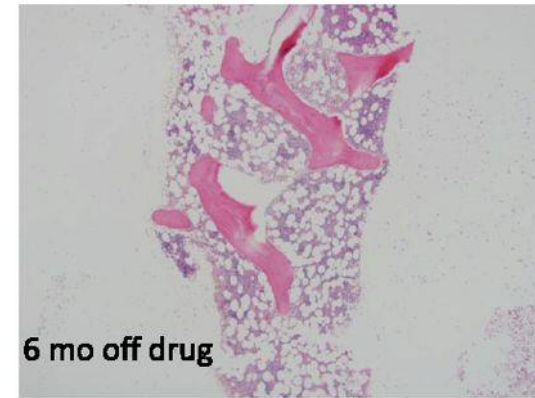
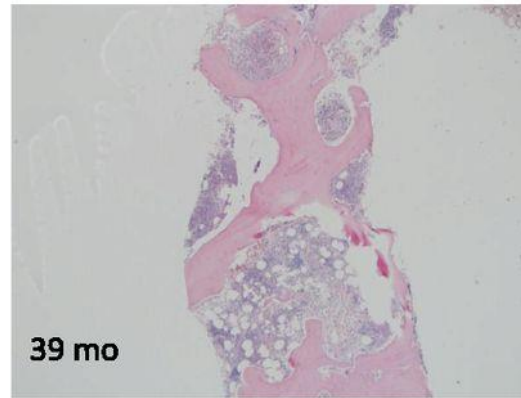
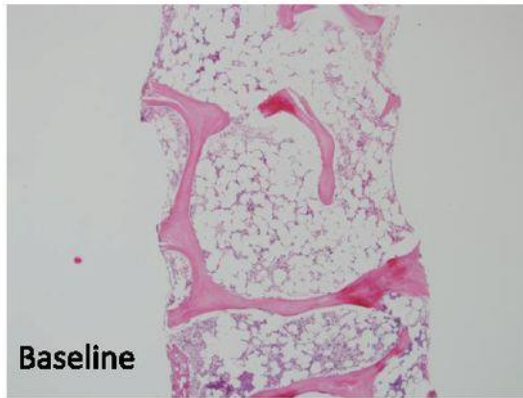
What if neither of these is suitable, or if IST fails?

NEW THING: ELTROMBOPAG

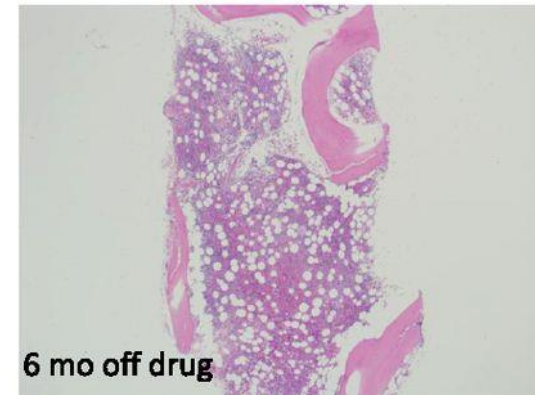
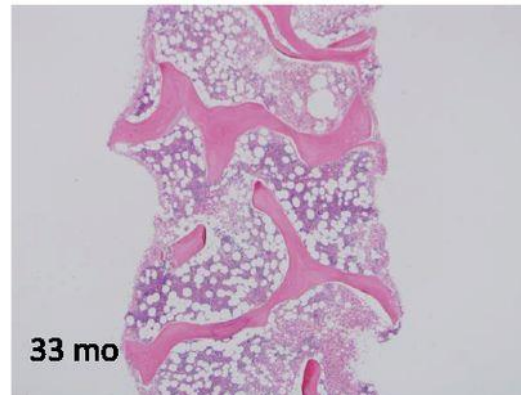
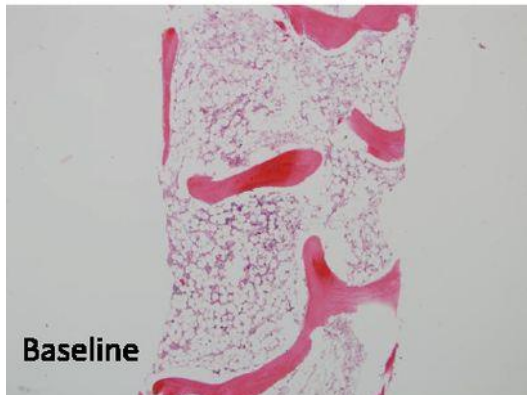
ELTROMBOPAG WORKS IN AA

40% responded

Patient 1



Patient 2



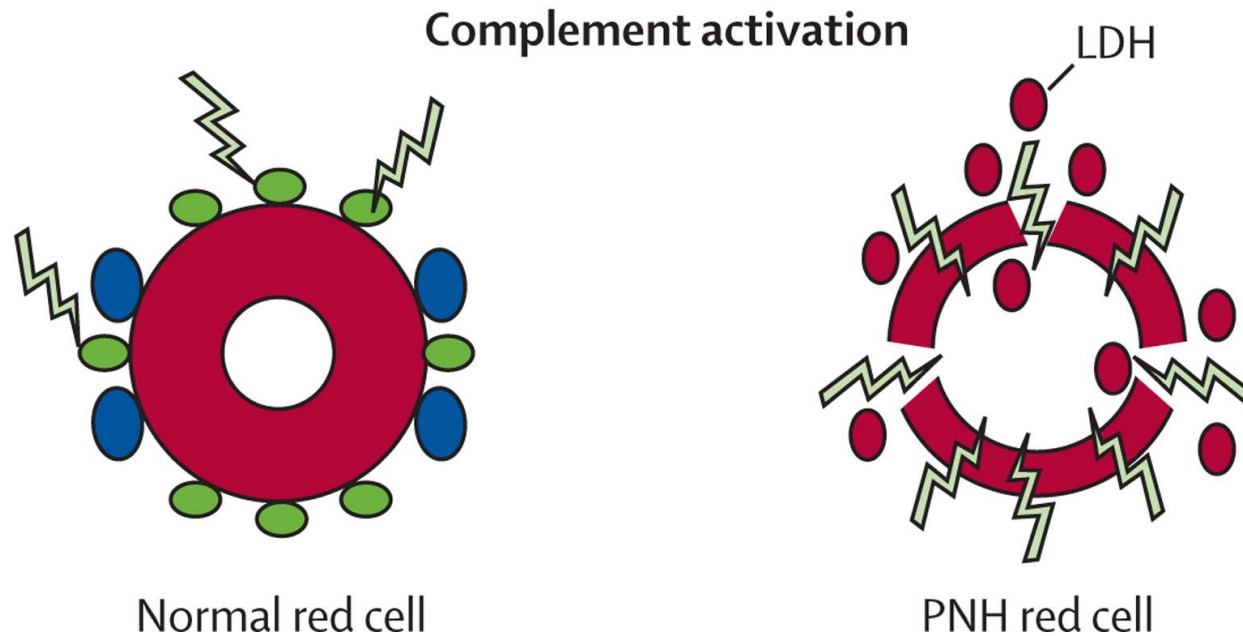
APLASTIC ANEMIA

Another new thing:

Health care system in crisis!

PNH

HOW PNH WORKS



Eculizumab (Soliris) blocks complement

PNH

Eculizumab is fabulous treatment

Do we need anything new?

Longer-acting?

Oral?

SUMMARY

We now have more treatments for MDS, AA, and PNH than ever before

There are still some gaps and areas where we need improvement

New drugs are being developed, and clinical trials will show us what works best

ACTIVISM HELPS!

Let your MPP know you expect fair and just provision of health care!