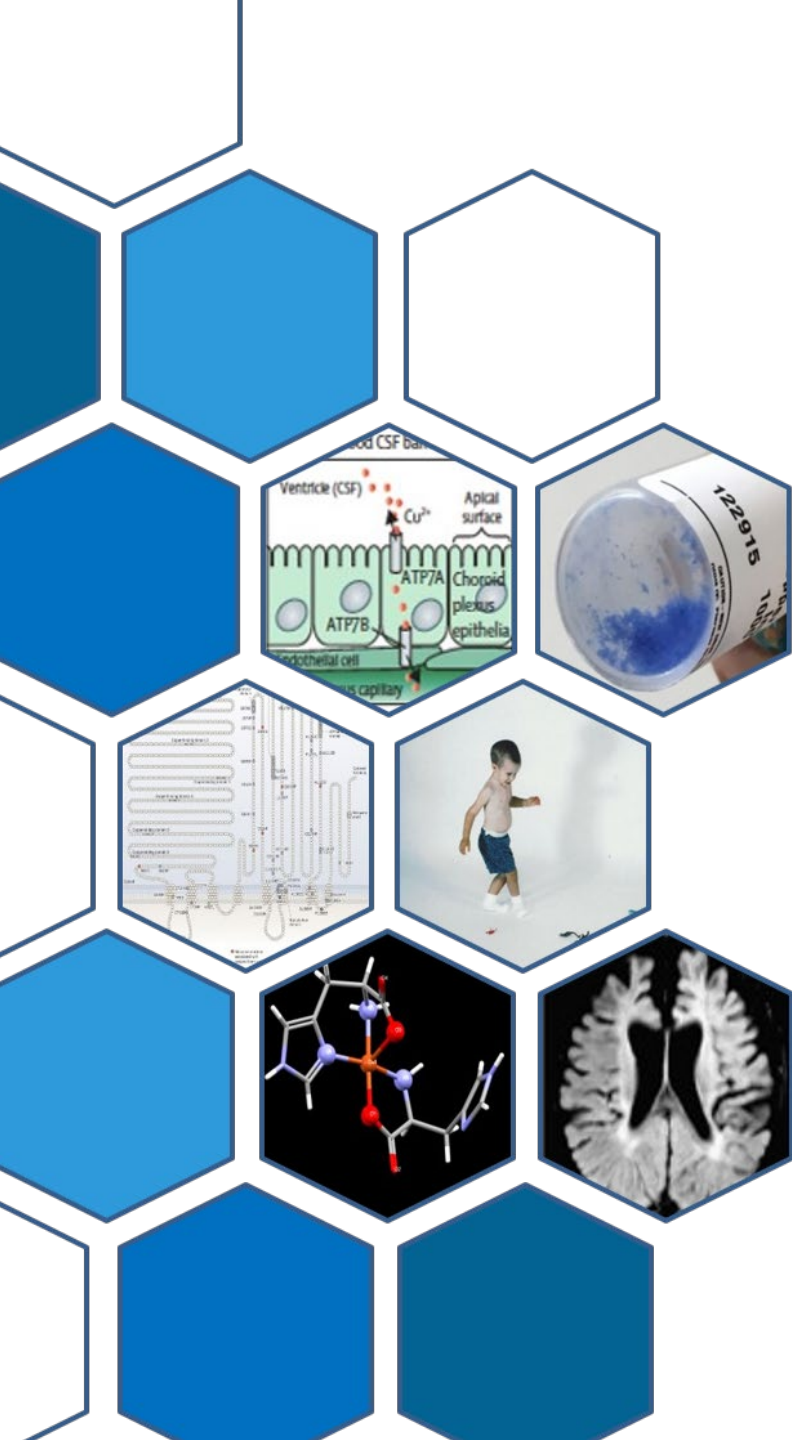


CYPRIUM THERAPEUTICS

Corporate Presentation

November 2022

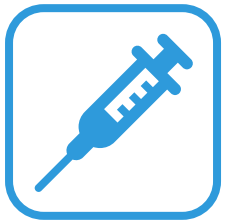
Forward Looking Statements

Some statements in this presentation that are not descriptions of fact may be forward-looking statements, for which we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, any statements relating to our growth strategy, products and product development programs. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, and financial condition. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; government regulation; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; risks relating to research, development and manufacturing activities; uncertainties relating to preclinical and clinical testing; risks relating to regulatory approvals and eligibility of our product candidates under certain government regulations; risks pertaining to drug safety; our dependence on third-party suppliers; our ability to attract, integrate, and retain key personnel; our need for additional funds; patent and intellectual property matters; competition; as well as other risks described in the Securities and Exchange Commission filings of our parent, Fortress Biotech, Inc. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as may be required by law. The information contained herein is intended to be reviewed in its totality, and any stipulations, conditions or provisos that apply to a given piece of information in one part of this presentation should be read as applying mutatis mutandis to every other instance of such information appearing herein.

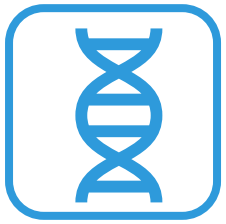
Cover Page Image Sources: Kaler SG, NIH

Company Highlights

- **Cyprium Therapeutics** is an orphan disease company with a focus on the development and commercialization of novel therapies for Menkes disease, a rare and fatal pediatric disease in copper metabolism.



- **CUTX-101 (Copper Histidinate Injections)** ODD FT RPD BTD OMP
  - **Rolling NDA submission to the FDA initiated in December 2021 – NDA submission expected to complete in 2023.**
  - **Potential to be the first FDA-approved treatment for Menkes Disease**
  - **Reported positive topline clinical efficacy data, showing a nearly 80% reduction in the risk of death (Hazard Ratio = 0.21, p<0.0001). Recent data presentation at AAP 2021 and ACMG 2022**
  - Intermediate-size Expanded Access protocol ongoing
  - FDA granted Breakthrough Therapy, Orphan Drug, Fast Track, and Rare Pediatric Disease Designations → Eligible for the Rare Pediatric Disease Priority Review Voucher.
  - EMA COMP granted Orphan Medicinal Product Designation
  - Development and Asset Purchase Agreement signed with Sentynl Therapeutics in February 2021



- **AAV-ATP7A Gene Therapy** ODD
  - Preclinical and already has Orphan Drug Designation from FDA
  - **Expects to nominate candidate for clinical development in 2023**

Copper is Required in Human Development and Health

Biological Functions	Copper Containing Proteins
Brain Development	
Catecholamine production	Dopamine β-hydroxylase
Mitochondrial respiration	Cytochrome C oxidase
Iron and copper transport	Ceruloplasmin
Peptide amidation	Peptidylglycine α-amidating monooxygenase
Antioxidant defense	Superoxide dismutase
Connective tissue formation	Lysyl oxidase
Pigment formation	Tyrosinase

Source: de Bie, et al, 2007; Image source: freepik.com

Menkes Disease is a Rare Pediatric Disease

Menkes Disease

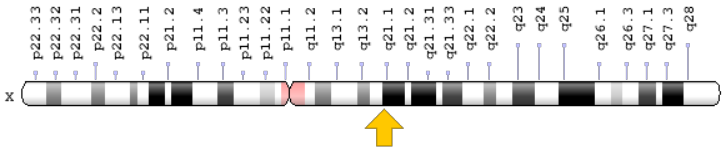
- First described by Dr. John Menkes in 1962
- X-linked recessive disease: affecting mostly boys
- Minimum birth prevalence for Menkes disease believed to be 1 in 34,810 live male births, but could potentially be as high as 1 in 8,664 live male births, higher than previously recognized
- Disorder of copper metabolism caused by mutations in the Copper transporter ATP7A
- **If untreated, premature death ~ 3 years**

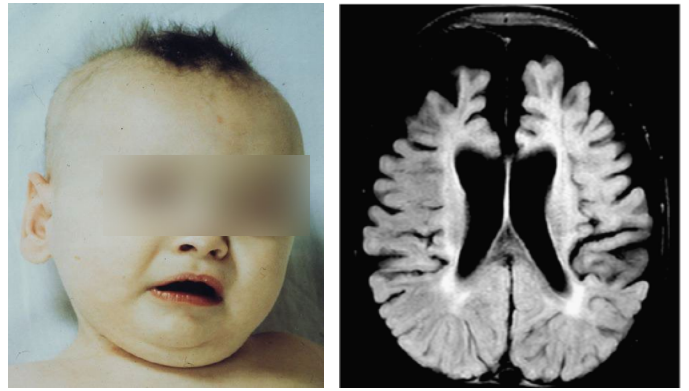
Distinctive clinical phenotypes

- Sparse, depigmented hair ("kinky hair")
- Onset of neurologic symptoms: seizures, hypotonia, and developmental delays
- Failure to thrive
- Connective tissue problems

Diagnosis

- Low serum copper and ceruloplasmin levels
- Abnormal catecholamine levels
- ATP7A gene sequencing confirmation





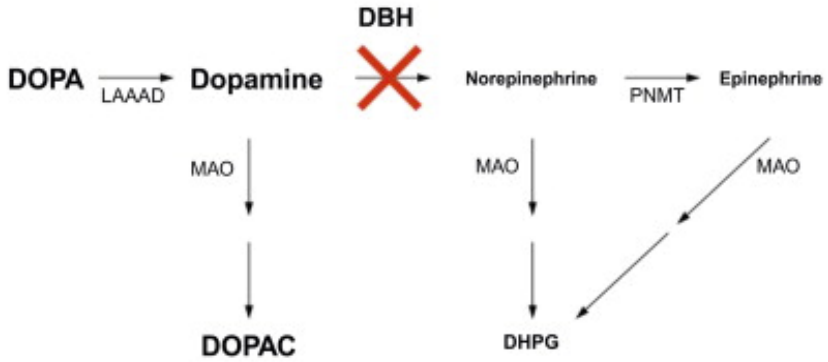
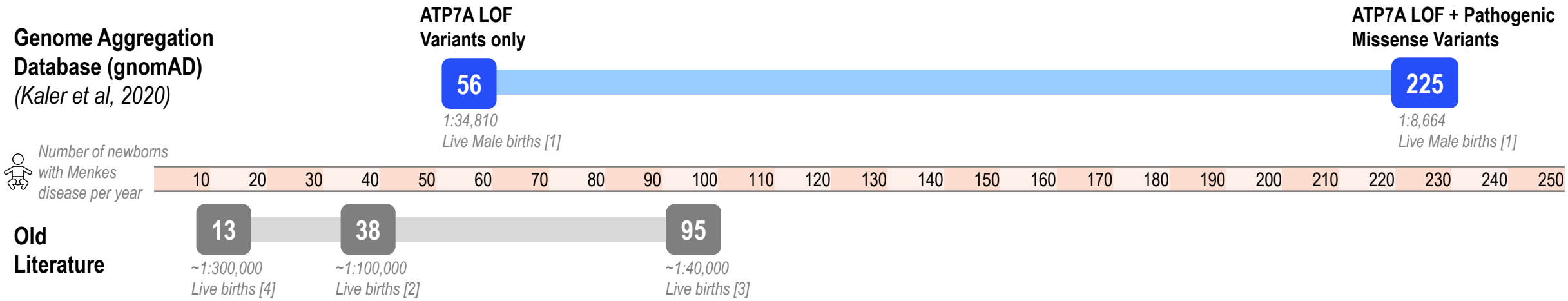


Image Source: Stephen G. Kaler, MD; NCBI;

Menkes Disease is Under-estimated and Under-diagnosed

New study estimated birth prevalence of Menkes disease based on the Genome Aggregation Database

- Accessed Genome Aggregation Database (gnomAD) at MIT/Broad Institute → over 200,000 ATP7A alleles
- Identified 1,106 ATP7A variants
  - 4 Loss-of-Function (LOF) variants → 4 alleles → 1:34,810 live male births → **56 patients per year**
  - 28 potentially pathogenic missense variants (PolyPhen-2) → 12 alleles with high confidence (REVEL >0.85)
  - Including both LOF and pathogenic missense variants → 1:8,664 live male births → **225 patients per year**
- Newborn screening (NBS) will likely increase the number of Menkes disease patients identified and allow early diagnosis and treatment with CUTX-101



References: [1] Kaler, et al, 2020; [2] Kaler, SG, 1998; [3] Danks DM, 1971; [4] Tonnesen et al 1991

Potentials of gnomAD and Newborn Screening to Discover More Patients in Rare Pediatric Diseases

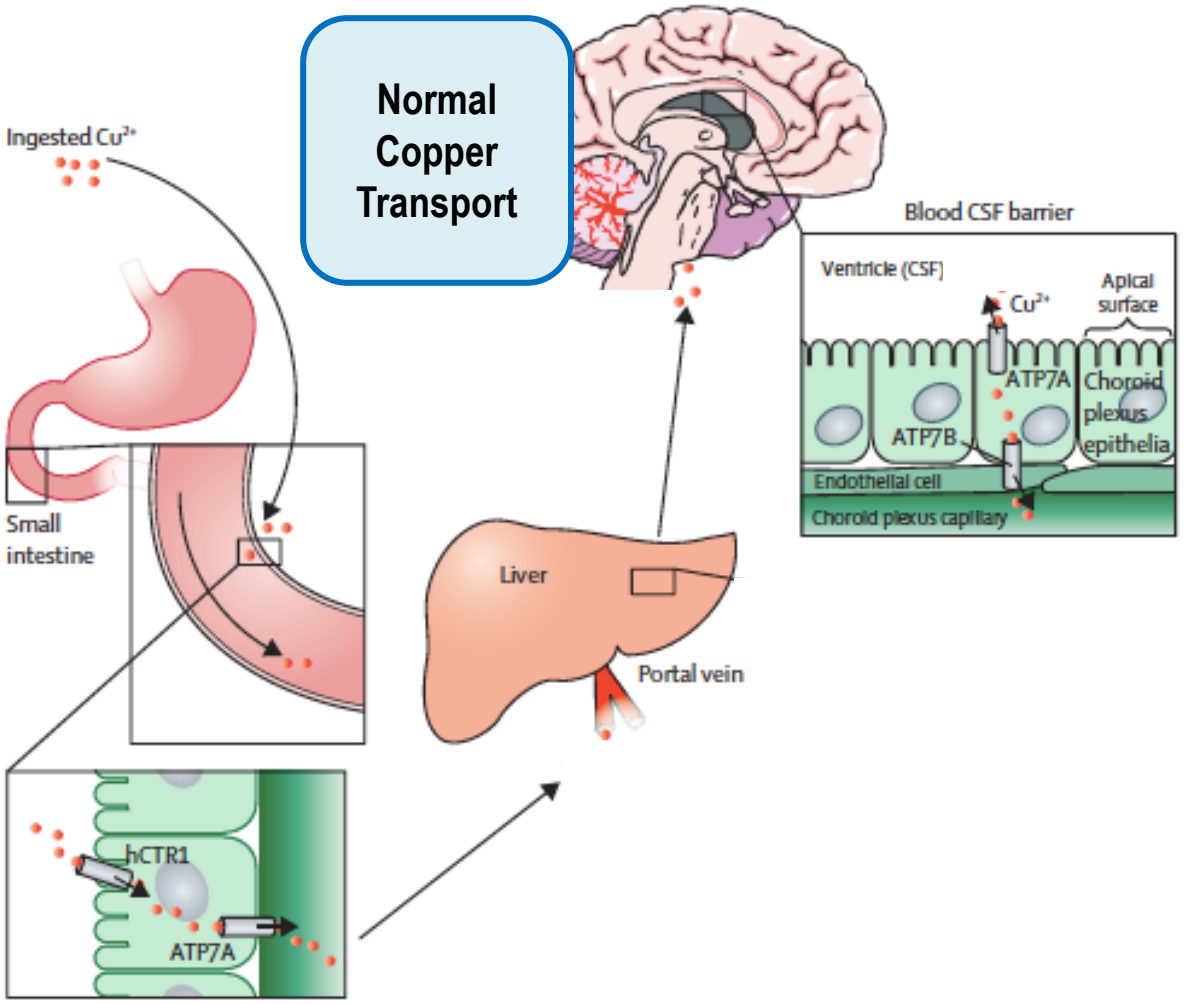
- Kaler 2020 study applied the same approach to a different X-linked recessive disorder, Duchenne Muscular Dystrophy (DMD), for which incidence data are better established due to longer lifespan.

- Analysis of gnomAD database entries for the DMD locus indicated 19 unequivocally loss-of-function alleles out of a total of 204,738 sequenced → predicted birth prevalence of DMD equals 1 in 7,246 live male births, in reasonable agreement with population-based estimates (1 in 5,000 newborn males) [1]

- Newborn screening detected a higher than previously estimated prevalence:
  - **Fabry Disease:** 1 in 8,454 in NBS [2] vs 1 in 40,000 to 60,000 males [3]
  - **Pompe Disease:** 1 in 21,979 in NBS [2] vs 1 in 40,000 births [4]

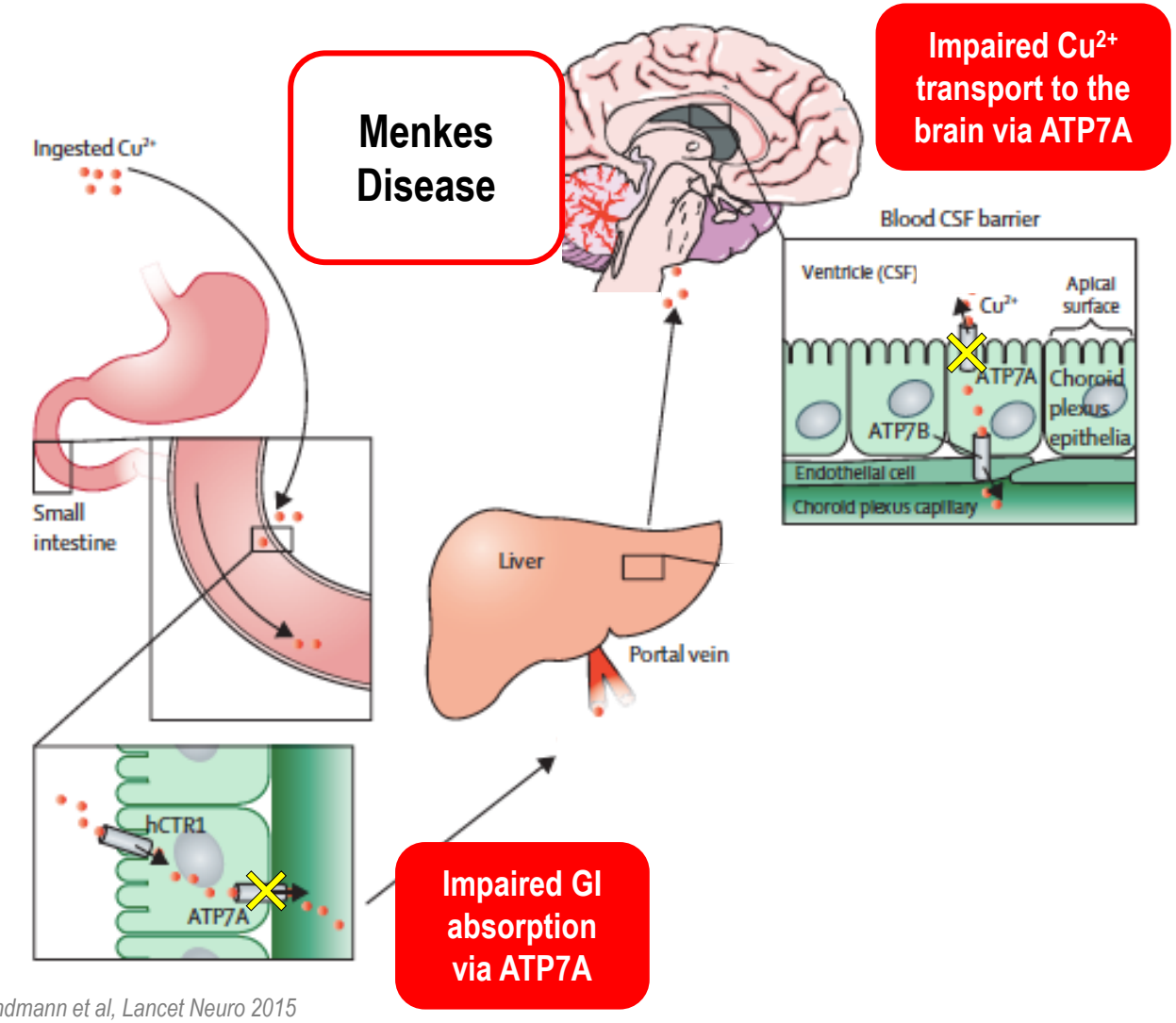
References:
[1] Kaler, et al, 2020
[2] Burton et al, 2017
[3] https://rarediseases.org/rare-diseases/fabry-disease/
[4] https://rarediseases.org/rare-diseases/pompe-disease/

ATP7A is Critical for Copper Transport to the Brain & GI



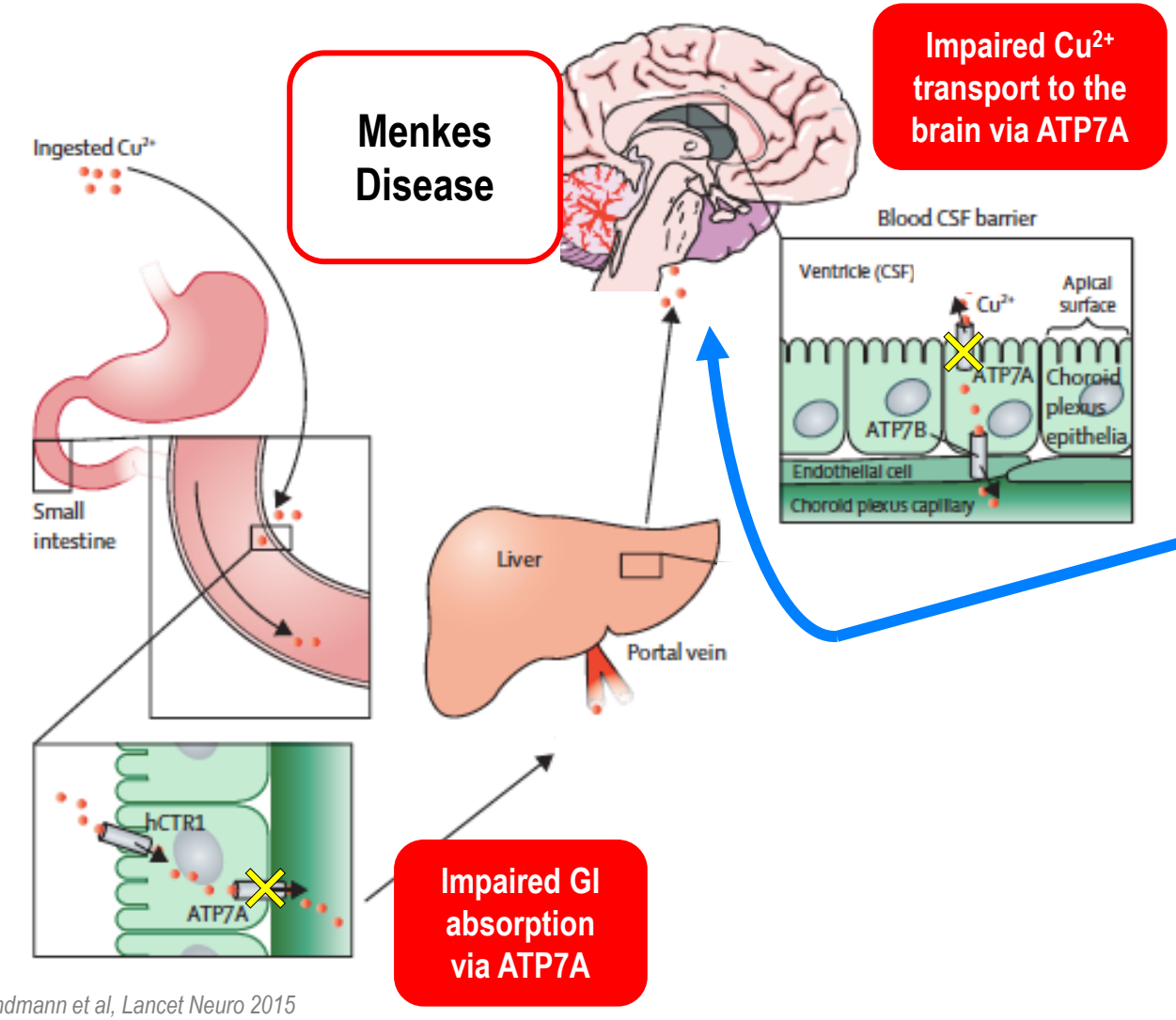
Adapted from: Bandmann et al, Lancet Neuro 2015

Copper Transport is impaired in Menkes Disease



Adapted from: Bandmann et al, Lancet Neuro 2015

Therapeutic Strategy for Menkes Disease: CUTX-101 (Copper Histidinate)



1

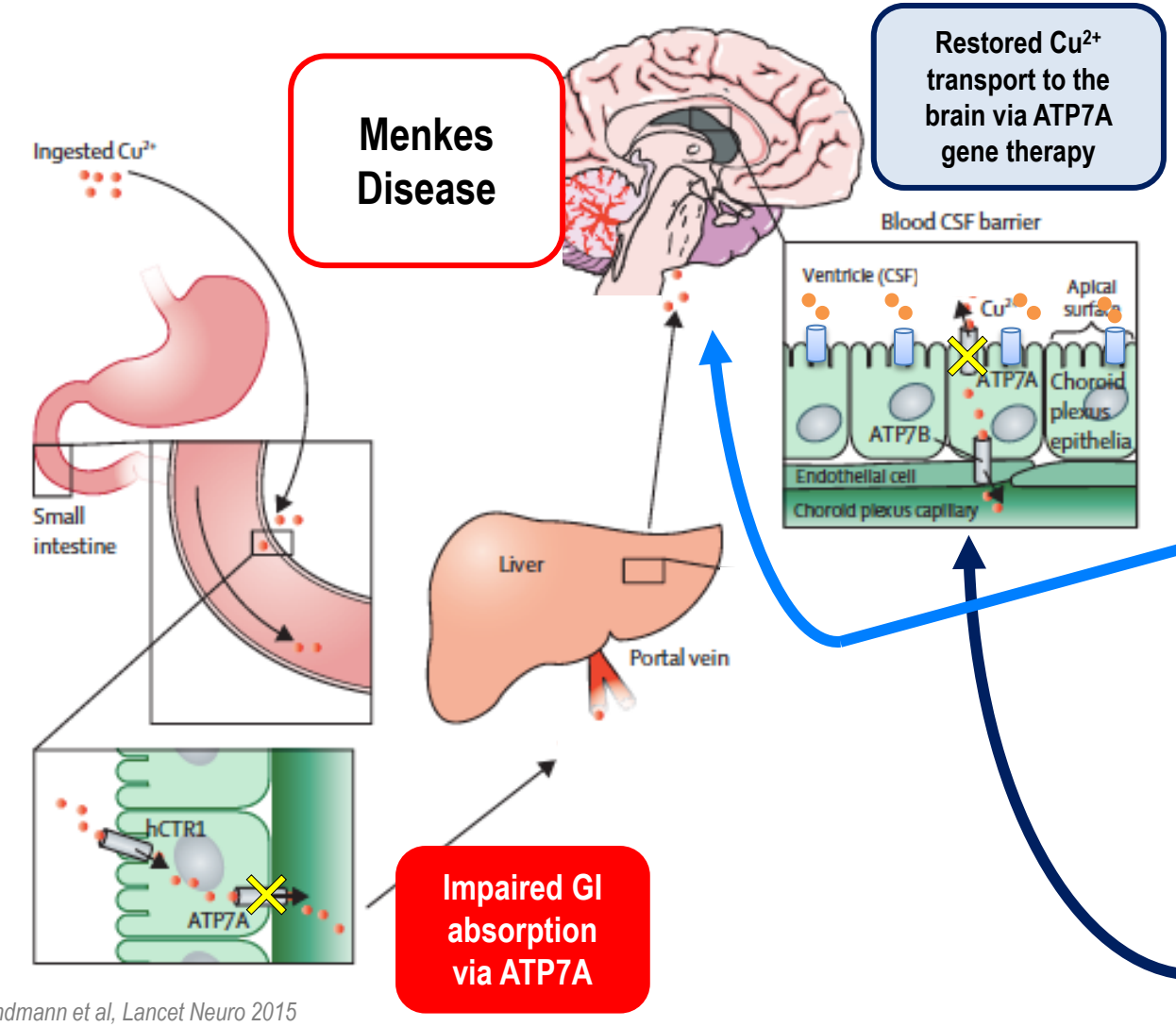
CUTX-101 Copper Histidinate

- SC injection to replenish CuHis
- Bypass GI absorption of Cu^{2+} (impaired in Menkes patients)
- Better tolerability (pH 7.4)
- May not be sufficient alone in some Menkes patients

- Reported positive topline clinical efficacy data
- Rolling NDA Submission initiated in December 2021
- Expanded Access protocol ongoing

Adapted from: Bandmann et al, Lancet Neuro 2015

Therapeutic Strategy for Menkes Disease: CUTX-101 (Copper Histidinate) + AAV-ATP7A Gene Therapy



Adapted from: Bandmann et al, Lancet Neuro 2015

1

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2

AAV-ATP7A Gene Therapy

- Codon-optimized reduced-sized ATP7A to be delivered via AAV vector (Preclinical)
- May restore Cu^{2+} transport
- Will need CUTX-101 injections

Preclinical

CUTX-101 is Optimized for Menkes Disease Patients

	CUTX-101 (Copper Histidinate)	Cupric Chloride ($CuCl_2$)	Oral Cu supplements
Route of Administration	Subcutaneous (SC)	IV (additive to TPN)	Oral
pH	~7.4 (physiologic)	2.0 (highly acidic)	N/A
Tolerability	Good	Poor (if injected SC)	N/A
GI absorption in Menkes patients	Bypassed	Bypassed	Very low
Bioavailability to cells	High	Low (Cu^{2+} ions bound to albumin)	N/A
Chemistry	Coordination complex (not free Cu^{2+} ions)	Inorganic salt (reactive free Cu^{2+} ions)	Inorganic salts
Clinical experience in Menkes patients	20+ years experience at NIH; 130+ patients treated	Minimal	Minimal

Product label; Deschamps et al 2005

Compelling Top-Line Clinical Efficacy Data for CUTX-101

	Early-Treatment (ET) Cohort		Late-Treatment (LT) Cohort	
	CuHis-ET (n=31)	Historical Control (HC-ET) (n=18)	CuHis-LT (n=35)	Historical Control (HC-LT) (n=17)
Median Overall Survival	**14.8 years (177.1 months)**	1.3 years (15.9 months)	**5.2 years (62.4 months)**	1.5 years (17.6 months)
Hazard Ratio (95% CI)	**0.208 (0.094, 0.463)**		**0.253 (0.119, 0.537)**	
p-value	**<0.0001**		**<0.0001**	
Reduction in Risk of Death	**79%**		**75%**	

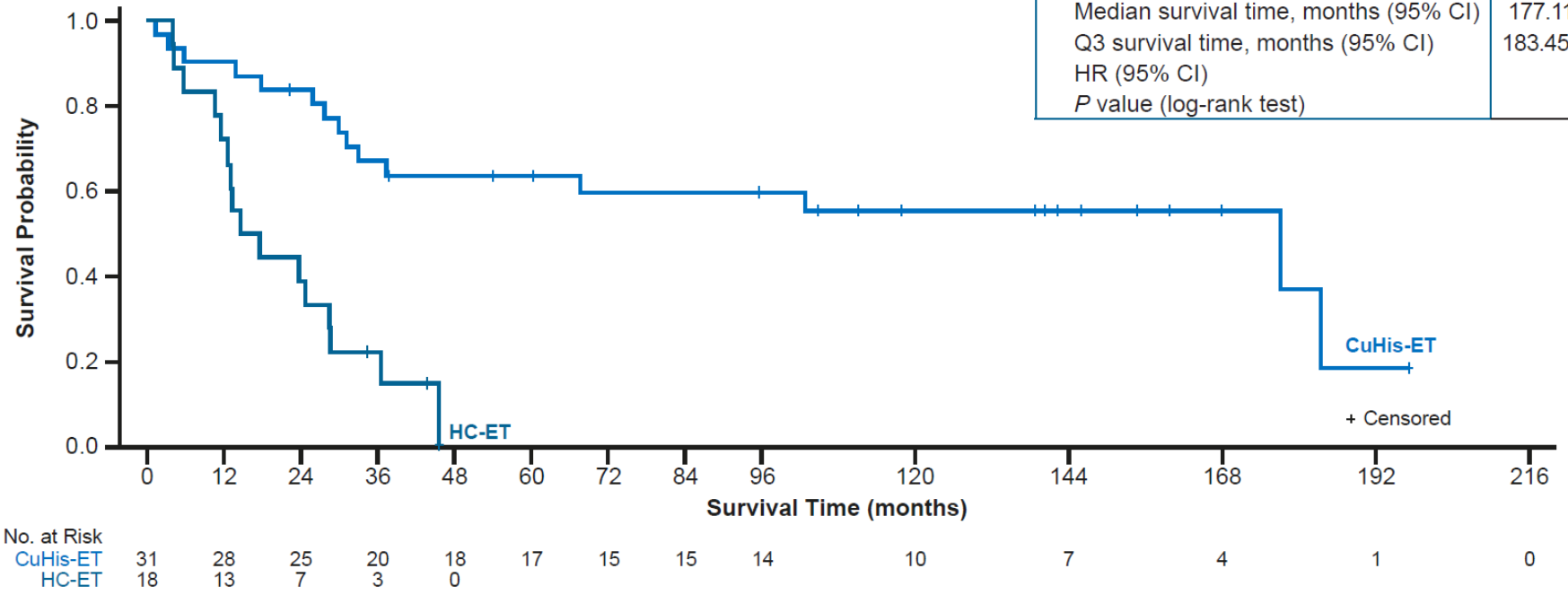
- CUTX-101 showed significant clinical benefit in both CuHis-ET and CuHis-LT cohorts, with 75-79% reduction in risk of death compared to untreated Historical Control (HC-ET and HC-LT) arms, and increase in Median OS from 1.3 years to 14.8 years in the ET cohort
- Newborn screening will be key to allow early diagnosis of Menkes disease and treatment with CUTX-101

Early-treatment cohort: initiated treatment with CUTX-101 within 4 weeks of age

Data presented as a virtual poster at the 2021 American Academy of Pediatrics (AAP) National Conference & Exhibition: Kaler SG, et al, Copper Histidinate Treatment for Menkes Disease (Kinky Hair Syndrome)

Kaplan-Meier Overall Survival Curves for Early Treatment Cohorts

	CuHis-ET (N=31)	HC-ET (N=18)
Subjects alive, n (%)	16 (51.6)	2 (11.1)
Q1 survival time, months (95% CI)	29.88 (5.75–67.66)	11.54 (3.98–13.28)
Median survival time, months (95% CI)	177.11 (32.98–NE)	16.08 (11.54–28.47)
Q3 survival time, months (95% CI)	183.45 (177.11–NE)	28.64 (17.62–45.63)
HR (95% CI)	0.208 (0.094–0.463)	
P value (log-rank test)	<0.0001	



NE, not estimable; Q1, quarter 1; Q3, quarter 3

- Data presented as a virtual poster at the 2021 American Academy of Pediatrics (AAP) National Conference & Exhibition:
  Kaler SG, et al, Copper Histidinate Treatment for Menkes Disease (Kinky Hair Syndrome)

CUTX-101: Current Status & Next Steps

Regulatory:

- **Rolling NDA submission initiated in December 2021 – NDA submission expected to complete in 2023**
  - **Potential to be the first FDA-approved treatment for Menkes Disease**
- FDA has been very helpful in providing guidance for regulatory pathway towards NDA submission for CUTX-101. FDA recommended Cyprium to continue frequent communications (Fast Track Designation, Breakthrough Therapy Designation)
- NDA is based on data from NIH studies and historical control, using overall survival as the primary endpoint
- **FDA granted Breakthrough Therapy Designation in December 2020**
- **EMA COMP granted Orphan Medicinal Product Designation in 2020**
- Additional regulatory activities in US and other territories

Clinical:

- Cyprium's Intermediate-Size Expanded Access Protocol CYP-001 (NCT04074512)
  - Provides CUTX-101 for newly diagnosed Menkes disease patients and patients from NIH study

CMC:

- Continue GMP manufacturing of CUTX-101
- Additional CMC and product development activities

Others:

- Additional PK and nonclinical studies to be completed based on FDA communications

Development and Asset Purchase Agreement signed with Sentynl Therapeutics

Development and Asset Purchase Agreement signed with Sentynl Therapeutics in February 2021

- Sentynl to acquire CUTX-101 for up to $20M in upfront and regulatory milestone payments through NDA approval
  - $8M was received upon signing with $12M remaining
  - Sales milestones totaling up to $255M
  - Royalties on CUTX-101 net sales:
    - 6% due on portion of annual net sales up to $75M
    - 17.5% due on portion of annual net sales between $75M and $100M
    - 25% due on portion of annual net sales over $100M.

- Cyprium will retain 100% ownership over any FDA Priority Review Voucher that may be issued at NDA approval for CUTX-101 with recent data suggesting PRVs transact for ~$100M to ~$110M

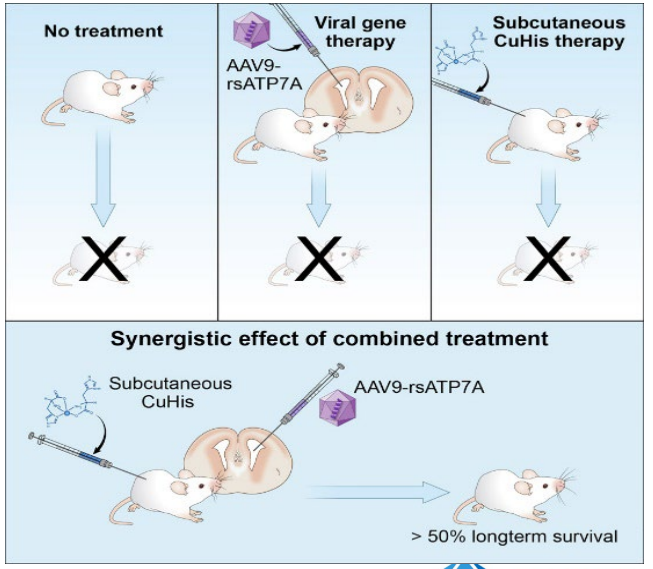
PRV Sale Amount ($M)	PRV Granted Drug	Approval Month	Sale Month	Seller
$110M	Vosoritide	Nov 2021	Feb 2022	BioMarin
$110M	Maralixibat	Sep 2021	Dec 2021	Mirum Pharma
$105M	Odevixibat	Jul 2021	Sep 2021	Albireo Pharma
$105M	Plasminogen, human-tvmh	June 2021	Aug 2021	Liminal BioSciences
Not Disclosed	Vitolarsen	Aug 2020	Jun 2021	Nippon Shinyaku
$102M	Casimersen	Feb 2021	Feb 2021	Sarepta Therapeutics
$100M	Setmelanotide	Nov 2020	Jan 2021	Rhythm Pharma
$105M	Naxitamab-gqgk	Nov 2020	Dec 2020	y-mAbs

PRV transactions based on public filings and press releases

AAV-ATP7A Gene Therapy for Menkes Disease

- *Mottled – brindled* mouse model recapitulates the disease phenotype
  - Atp7a$^{mo\text{-}br}$ phenotype
  - A 6 bp in-frame deletion in exon 11 of Atp7a
  - Depigmented coat color and curly whiskers
  - Premature death (~13 days of age)
  - Poor growth; Neurological symptoms
  - Low brain copper; Abnormal catecholamine levels

- NICHD has developed several constructs for reduced size, codon-optimized AAV-ATP7A gene therapy
- AAV-ATP7A + SC CuHis administration led to:
  - Improvements in muscle strength, balance and coordination in preclinical model
  - Improved biochemical phenotype (Cu and catecholamine)
  - Improved survival



Source: Stephen G. Kaler, MD; Haddad et al, 2018

Thank you!

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