

Argomenti della presentazione:

CF Europe:

- LIFC nei gruppi di lavoro di Ricerca della CF Europe
- CTN Expansion Competitive Renewal Project
- Right to Breath (accesso ai farmaci)



Congresso Boston USA NACFC 2024:

- Argomenti Plenarie
- Nuova triplice di Vertex
- Nuovi Trials in arrivo

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LIFC nei Gruppi di lavoro di Ricerca della CF Europe

Community Advisory Board (CAB)



Mario Ricciardi, PhD : Paziente FC, Regulatory Consultant

Il CAB è un gruppo di rappresentanti qualificati dei pazienti provenienti da 13 paesi diversi che partecipa a riunioni riservate con le aziende per esplorare questioni specifiche di ciascuna azienda.

Le riunioni consentono di delle comunità di pazienti affetti da FC di dare consigli imparziali alle aziende al fine di ottimizzare la ricerca e lo sviluppo per soddisfare al meglio le esigenze e le preferenze dei pazienti.

Patient Organisations Research Group (PORG)

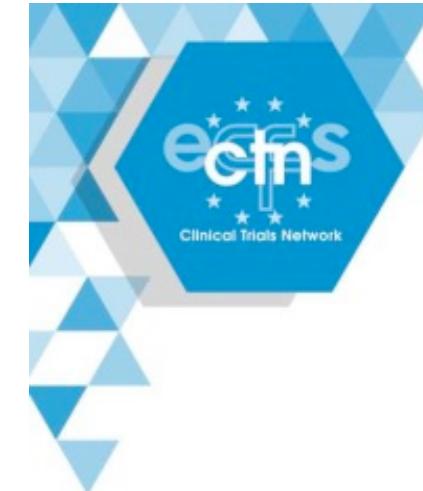


dott. Giuseppe F. Parisi, PhD Pediatra Centro FC Catania

L'obiettivo del PORG è quello di migliorare le partnership tra le organizzazioni europee associate, al fine di espandere la rete europea di ricerca sulla FC e accelerare l'accesso a nuovi farmaci per le persone con FC attraverso la ricerca.

Il PORG conta attualmente 11 membri, tra cui Belgio, Francia, Germania, Irlanda, Israele, Italia, Lussemburgo, Paesi Bassi, Polonia, Svizzera e Regno Unito.

Timeline	Competitive renewal	Expansion
April	ECFS-CTN sites complete online re-application form. French subsites re-apply as standalone sites. Applications close Friday April 26th	
May	Review panel scores sites and suggests which (if any) sites should leave network	Expansion announced, eligibility & selection criteria explained, and application opened
June	Executive Committee and ECFS Board review & confirm outcome of competitive renewal	Expansion publicised at Glasgow conference
July	Outcomes communicated to all ECFS-CTN sites	Applications close
August		Applications are checked for eligibility
September	Low-performing sites leave network at end of month (aligning with end of CRC funding)	Review panel score & rank applications
October		
November		Executive Committee and ECFS Board review & confirm outcome of expansion
December		Applicants notified of outcome
January 2025		New sites join Steerco meeting



- 5 Past CTN directors
- CFF-TDN representative
- CanACT representative
- European Patient Organisation representative (LIFC)

ECFS-CTN Competitive Renewal Project 2/2

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RE: ECFS-CTN review applications expansion - Group 1 ➤ Posta in arrivo x

→ European Clinical Trial Network

a me, European ▾

Traduci in italiano

X

Dear Marco,

Many thanks for sending in the scoring forms. It is clear that you have put a lot of work into this.

Thank you so much for having helped with this, much appreciated.

The further evaluation steps could take some time now, we should have a final outcome in December.

Wishing you a nice evening!

Kind regards,

Anne

Anne VERBRUGGE

Project Manager

ECFS - Clinical Trials Network

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www.ecfs.eu/ctn



Sign on: Cystic Fibrosis & Respiratory Orgs Support CF Treatments' Inclusion on the WHO Essential Medicines List (EML).



Thank you for supporting the global cystic fibrosis community's efforts to overcome the inequalities in access to lifesaving CFTR modulator treatments.

www.righttobreathe.net

L'elenco dei farmaci essenziali dell'Organizzazione Mondiale della Sanità identifica i farmaci più importanti necessari per affrontare le principali esigenze di salute pubblica a livello globale.

Questi farmaci vengono scelti in base alla loro efficacia, sicurezza ed economicità, con l'obiettivo di garantire che i trattamenti essenziali siano accessibili a tutti, soprattutto in contesti con scarse risorse.

L'inserimento di farmaci ad alto costo nell'elenco dei farmaci essenziali è vantaggioso perché può contribuire a far scendere i prezzi attraverso acquisti all'ingrosso, negoziazioni o la promozione di farmaci generici. Pertanto, l'inserimento dei modulatori CFTR nell'elenco è un passo importante per ottenere l'accesso globale a questi farmaci.

Inoltre, l'elenco dei farmaci essenziali evidenzia la priorità sanitaria globale di garantire che i farmaci salvavita, indipendentemente dal costo, siano disponibili e accessibili a tutti coloro che ne hanno bisogno.



www.righttobreathe.net

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BOSTON | SEPTEMBER 26-28



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PL1--Challenges & Progress in the Pursuit of Genetic Therapies for Cystic Fibrosis

PL2--The Tipping Point: The Journey to Understand & Advance Research in CFRD

PL3--Reproductive Health in Cystic Fibrosis



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Hope in the CF Community

"The incredible advancements in CF research. There is hope for a brighter future ahead."

Person with CF

"Seeing so many of us defy the odds... graduate, get jobs, get married, have children."

Person with CF

"Seeing my patients on the path to living fulfilled lives."

CF Clinician

"Knowing that everything that we as a community are doing now is going to benefit the future generation and show them what is possible."

Person with CF

"Research, new medicine, and the rise of life expectancy!"

Parent of a person with CF

"My CF [care] team! Not only do they fight for me and with me, they cheer me on every step of the way!"

Person with CF

"Clinical trials for the roughly 10% of CF patients with nonsense mutations."

Person with CF

"That even the last .0001% like my daughter will have a cure."

Parent of a person with CF



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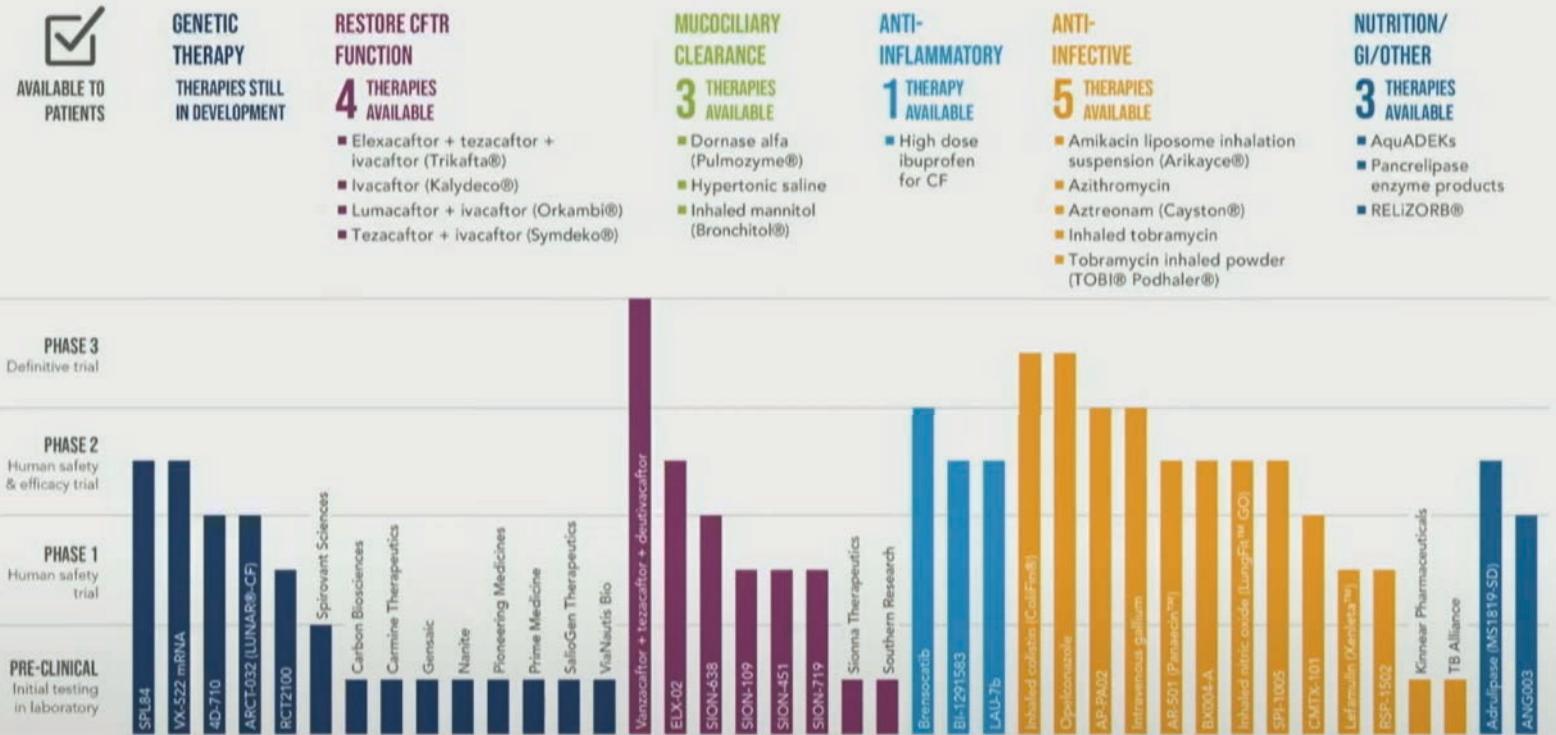
PL1--Challenges & Progress in the Pursuit of Genetic Therapies for Cystic Fibrosis

- Identificare le sfide biologiche dello sviluppo di una terapia genetica per la fibrosi cistica.
- Identificare le innovazioni nelle tecnologie di somministrazione dei farmaci e come queste possano supportare una varietà di terapie genetiche.
- Descrivere come le terapie genetiche abbiano il potenziale per ripristinare la funzione CFTR nei polmoni, indipendentemente dalla mutazione CFTR sottostante.
- Esprimere come gli studi clinici per le terapie genetiche richiedano un'attenta progettazione degli studi clinici e un maggiore coinvolgimento della comunità.

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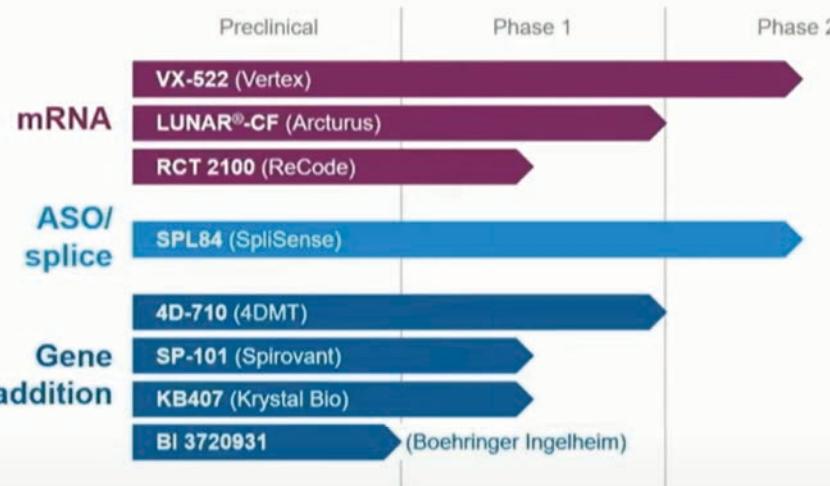
DRUG DEVELOPMENT PIPELINE



To advance drug development and a search for a cure, the Cystic Fibrosis Foundation (CFF) has contracts with several companies to help fund the development of potential treatments and/or cures for cystic fibrosis. Pursuant to these contracts, CFF may receive milestone-based payments, equity interests, royalties on the net sales of therapies, and/or other forms of consideration. Resulting revenue received by CFF is used in support of our mission. See "How Drugs Get on the Pipeline" at www.cff.org/howdrugsgetonthipeline for more.

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Early phase genetic therapies



Critical need:

- Recruitment from restricted eligible population

Terapia genica

fornisce all'organismo una copia corretta di un gene difettoso o un altro gene che possa compensarne il malfunzionamento

mRNA

fornisce alla cellula le informazioni corrette per sintetizzare la proteina CFTR normale

Editing genomico

manipolazione genetica in cui si procede alla delezione, all'inserimento, alla sostituzione o alla modifica del DNA genomico di un organismo vivente

Oligonucleotidi antisenso

piccoli pezzi di DNA o RNA che si legano alla molecola di RNA e correggono queste istruzioni in modo da poter produrre una proteina CFTR a lunghezza intera

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PL2--The Tipping Point: The Journey to Understand & Advance Research in CFRD

Descrivere i complessi processi cellulari che contribuiscono allo sviluppo della CFRD.

Identificare nuove promettenti ricerche volte a migliorare la comprensione del pancreas FC.

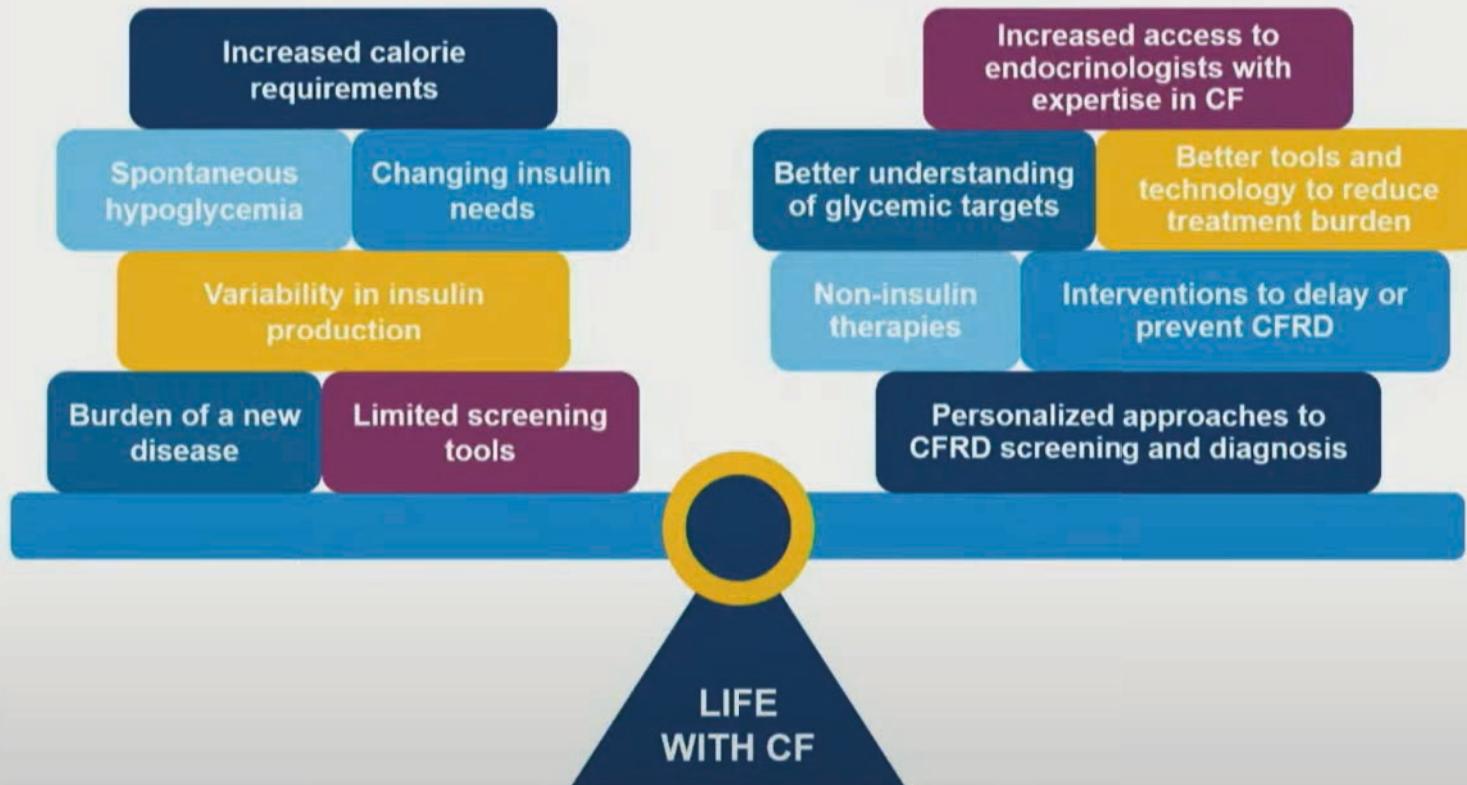
Descrivere le opportunità di migliorare lo screening, la diagnosi e il trattamento della CFRD utilizzando le nuove tecnologie per il diabete.

Descrivere gli studi clinici in corso e futuri che cercano di ampliare le opzioni di trattamento, ridurre l'onere, migliorare l'accesso e informare le migliori pratiche nell'era post-modulatore.

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The future of CFRD: Shifting the tipping point



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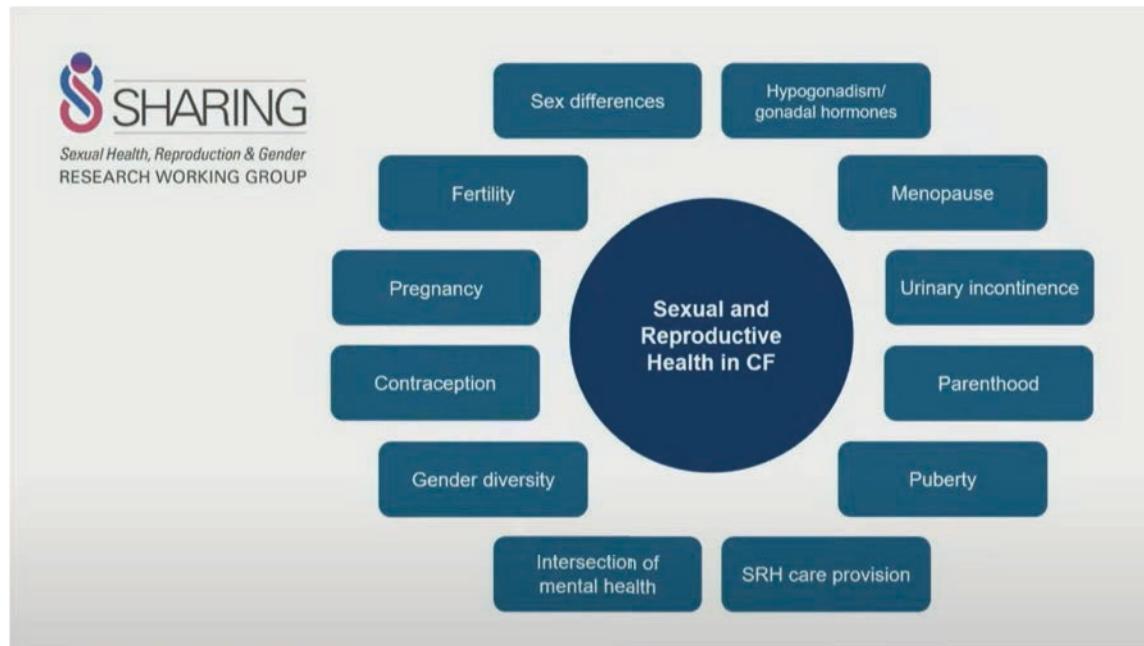


PL3--Reproductive Health in Cystic Fibrosis

Discutere le dinamiche di cambiamento della fertilità e della gravidanza per le donne con FC e le potenziali implicazioni per i loro bambini nell'era moderna.

Esaminare l'infertilità maschile e il viaggio attraverso la tecnologia di riproduzione assistita.

Riassumere le ricerche in corso sull'interazione tra FC e salute riproduttiva e i potenziali interventi clinici per migliorare l'assistenza.

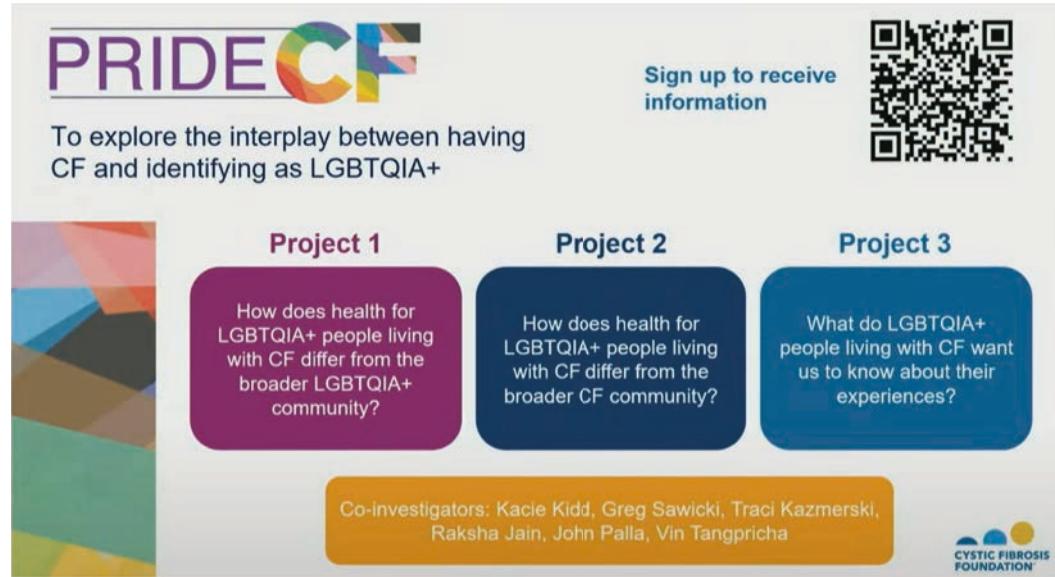


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Community Survey: SRH Priorities

Top topics that
should be prioritized
for CF SRH research
(n=237)

- Fertility
- Pregnancy
- Assisted reproductive technology
- Effects of sex hormones on CF
- Sexual functioning



PRIDE CF
To explore the interplay between having CF and identifying as LGBTQIA+

Project 1
How does health for LGBTQIA+ people living with CF differ from the broader LGBTQIA+ community?

Project 2
How does health for LGBTQIA+ people living with CF differ from the broader CF community?

Project 3
What do LGBTQIA+ people living with CF want us to know about their experiences?

Co-investigators: Kacie Kidd, Greg Sawicki, Traci Kazmerski, Raksha Jain, John Palla, Vin Tangpricha

Sign up to receive information 

CYSTIC FIBROSIS FOUNDATION



Conclusions

- SRH is complex but increasingly important to people with CF
- We are seeing more pregnancies
- Ongoing studies will enable us to have more informed conversations
- Individual circumstances will still require shared decision making

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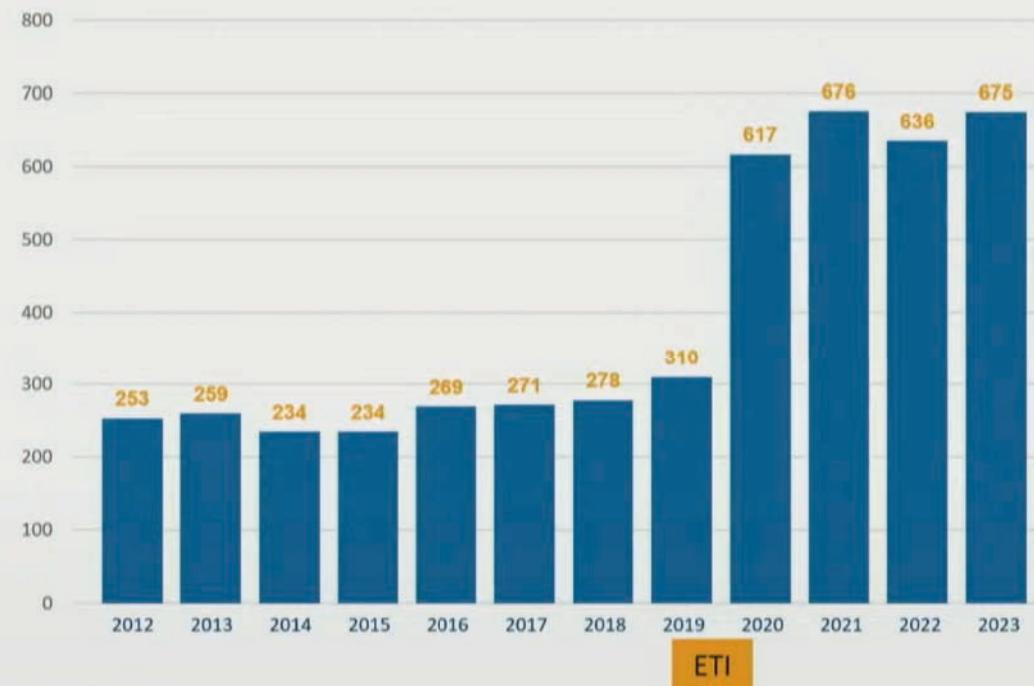


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Females: Baby Boom!

Number of pregnancies in females ages 14-45 with CF



Cystic Fibrosis Foundation Patient Registry 2022 and 2023 Annual Highlights Report

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People with CF want to discuss reproductive health with their CF teams but aren't

n>100 (interviews/focus groups)

"I don't think they [CF providers] think about vaginas. I just think they like to think about lungs."

"I have trouble going to other doctors that aren't my CF doctor because I feel ... it's all really CF-related."

"I mean [reproductive health] is a really big part of everyone's life, and there is a taboo about talking about it. It's just as important as other parts of your health that people talk about a lot more easily..."

Kazmerski et al, Pediatrics, 2016.
Leech et al, Contraception, 2020

Kazmerski et al, Pediatr Pulmonol, 2018
Clarke et al, J Cyst Fibros, 2022

Poster #120

Vanzacaftor/Tezacaftor/Deutivacaftor in Adolescents and Adults with Cystic Fibrosis: Results from Two Randomized, Active-Controlled Phase 3 Trials

Craig Keeling,¹ Laird M. Yorkin,² François Vermeulen,³ Dario Prati,⁴ Rachel W. Lommens,⁵ Aaron Trimble,⁶ Tom Kotarski,⁷ Joel Merris,⁸ Andrew T. Braun,⁹ Mark O'Carroll,¹⁰ Michael J. Kuehn,¹¹ Daniel J. O'Donnell,¹² Edward P. McKone,¹³ Elizabeth Laffel,¹⁴ Tim Farrow,¹⁵ Peter McNeilson,¹⁶ Patrick R. Salsbury,¹⁷ Ming Naix,¹⁸ Benjamin Y.Y. Yu,¹⁹ Hannah Martin,²⁰ Ned Abman,²¹ Anna Lam,²² Alexander Honig,²³ Claire Keeling,²⁴ Laird M. Yorkin,²⁵ François Vermeulen,²⁶ Dario Prati,²⁷ Rachel W. Lommens,²⁸ Aaron Trimble,²⁹ Tom Kotarski,³⁰ Joel Merris,³¹ Andrew T. Braun,³² Mark O'Carroll,³³ Michael J. Kuehn,³⁴ Daniel J. O'Donnell,³⁵ Edward P. McKone,³⁶ Elizabeth Laffel,³⁷ Tim Farrow,³⁸ Peter McNeilson,³⁹ Patrick R. Salsbury,⁴⁰ Ming Naix,⁴¹ Benjamin Y.Y. Yu,⁴² Hannah Martin,⁴³ Ned Abman,⁴⁴ Anna Lam,⁴⁵ Alexander Honig,⁴⁶ Claire Keeling,⁴⁷ Laird M. Yorkin,⁴⁸ François Vermeulen,⁴⁹ Dario Prati,⁵⁰ Rachel W. Lommens,⁵¹ Aaron Trimble,⁵² Tom Kotarski,⁵³ Joel Merris,⁵⁴ Andrew T. 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VX-522

- L'obiettivo è fornire una copia a lunghezza intera del CFTR mRNA alle cellule polmonari utilizzando come vettore una nanoparticella lipidica e non un Virus.
- Le cellule polmonari userebbero quindi le istruzioni contenute nel mRNA per creare la proteina CFTR funzionale.
- Questa terapia potrebbe funzionare per qualsiasi persona con FC, indipendentemente dalle sue mutazioni CFTR.



LUNAR

- LUNAR®-CF è una terapia sostitutiva dell'mRNA, per aerosol per il trattamento della malattia polmonare nella fibrosi cistica ed è indipendente dal genotipo.
- Un mRNA CFTR umano incapsulato in LUNAR®, una nanoparticella lipidica, è stato sviluppato per fornire l'mRNA nell'epitelio delle vie aeree. Le proprietà fisicochimiche di LUNAR® erano stabili dopo l'aerosol.



RCT 2100

- RCT 2100 ha un approccio simile a quello di LUNAR ma con l'obiettivo limitato alle mutazioni No Sense/Rare

Quello che fino a tre anni fa, mi sembrava una speranza, oggi diventa un obiettivo concreto, che penso potrà raggiungersi entro 5 anni con il rilascio di nuovi farmaci e terapie.



Grazie dell'attenzione !
magri.marco@gmail.com