Les essais cliniques	: présentation de Myriam Rai, FAR	, lors du webinaire du 19 novembre 2022
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Développeur de Fessal	Lexeo Therapeutics	Design Therapeutics	Larinar Thorapoutics	Children's Hospital of Philadelphia (CHO)	Resta Pharmacouticals	PSC Therapeutica  Variquinane (PTC-240)  Amiliare Regulière mitochondrial et cellulaire en cittart la 15-lipocoppinase, l'engyme cité de régulation de la ferroptose	
Composé	LX2006 (MANA-106FXN	01.216	CTL1601	NACT et exercice	Omeveloxokose (KTA- 408)		
Principe	Thirspe gérique par virus adéno-associé (AAV) pour la cardomyopather conque pour poter le géne de la flataxine humane aux cellules cardiaques. Pounsit attendre le cosur, le muscle sipoiethique et if autres trous perghériques.	Patites molécules, les 'Gene-TAC' qui ciblent l'expansion GAA et, en construisant 'un pont', ntactivent le gène de la flutavine	Protéine de fusion. Apporte la fistavore frumaine directement aux tissus par injection sous-cutanée.	NACH est converti en NACH dans la celtule. Ties important au sein de la mitochondrie pour produire de l'amergie. L'exercice augmente la masse musculaire, la capacité pulmonaire et cardiague et amiliore la fonction mitochondrale.	Active le NiQ (anti- oxydant cellulane) Augmente l'expression des pines « arti- oxydants ». Protège les cellules des dégits consèquents.		
Age des patients	Pour adultes présentant une cardiomyopathie	16 - 55 are	Adultes de plus de 19 aris	10 - 40 are	MCIDE 16-40 ats	MOVE FA: Enfants de plus de 7 ans et adultes	Extre 2 et 5 enfants d moins de 7 ans
Durée du traitement	12 senanes - admissible en 1 seule fois parvois intransmune.	Eserates	A samplines	2 semaines	Toujours en cours	Hess	72 semanes
Etat de disveloppement	Phase 10 Deux groupes faible dose et haute dose (5 patients dans chaque groupe). Pas de placebo. Deux centres suverta aux USA.	Pitace 1 doces croissantes multiples (MAC) pour évaluer la sécurité, la tolérabilité, la pharmacocinétique et la pharmacodynamique de 3 doces hebdomadaires chez des palients adubes.	Phase 2 pour détermner la dose et étuder la distribution de la fataxine externe et la tolerabilité.	Recrutement (seutement aus USA actuellement)	Attente d'approbation de la FCA (Agence américaine médicament), Sourcesson d'une demande d'autonaution de mise our le marché a l'EMA (Europe) fin 2022.	Phase 2/3 on cours, your dvaluer (efficacital et la sécurité	Racrutement, Les enfants premient la molécule active (pas e placebo). Un seul centre clinique aux USA, mais ils accepte des enfants étrangen si les parents sont capatites de comprendre le consentement éclaire et le signer.
Resultate / Décisions	2029	2023	2023	D464 2024	28 Notice 2023	Vws rs 2023	2024

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