# Alpha1 MZ Foundation - Information & Research

## **News & Research Update**

Jul 13, 2024

Dear Subscribers,

#### In this issue:

- 1. Update on our organization
- 2. Update on our own research
- 3. More on Therapeutic Medication

## **Organization update**

Because we are getting more and more involved with other Alpha1 related professional medical organizations, it was time to formalize our Alpha1 MZ Information & Research organization, and get incorporated. (In the chamber of commerce etc.) This means we are now officially a Foundation. You may have noticed the change already on the website, which is now Alpha1 MZ Foundation — Information & Research. It will not really change things, but it allows us to have a bank account, make payments etc.

## **Research Update**

We also like to inform you at this time, that we are making steady progress with our current research studies. As you may already know, the paper related to the MZ liver has been ready for a couple of months now, and we are currently preparing the study with patient cohorts together with other institutions. The preparations are quite intensive, and it just takes time to do this properly. The execution of the study may also take about 1 year to execute.

#### **Therapeutic Medication**

In the previous newsletters we covered already a couple of the upcoming therapeutic medication. In this issue we like to mention ADARx, which is a relative new company established in 2019 and is located in San Diego. In 2023 they raised about 200M\$ in series C, which shows the high interest among investors in therapeutic medication. (which is good for us..)

ADARx lead program (according to their information) is an RNA Editing therapeutic targeted for Alpha1 patients, and is in Phase Ib. They state:

"Through our RNA editing approach, we can specifically target an RNA sequence and, utilizing existing cellular machinery, precisely edit a single base. Our novel design is compact and stable resulting in efficient precision therapeutics. Our first editing program is directed to the treatment of Alpha-1 Antitrypsin Deficiency"

And like always, enjoy the ride !!

Our website https://www.alpha1mz.org

Facebook: https://www.facebook.com/groups/790526129640939/