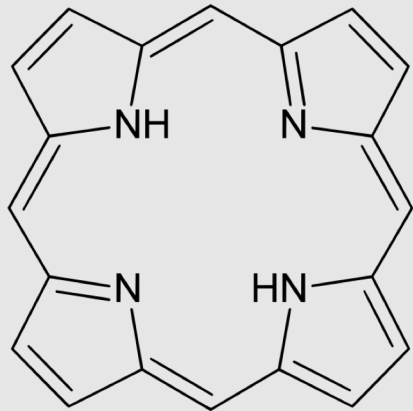


# Smartphone-based digital devices for quantitative disease monitoring in erythropoietic protoporphyria



Amy Dickey, MD  
Massachusetts General Hospital  
IDEA<sup>2</sup> Global Review Workshop  
October 18, 2019



# Disclosures

- I am on the advisory board for Alnylam Pharmaceuticals

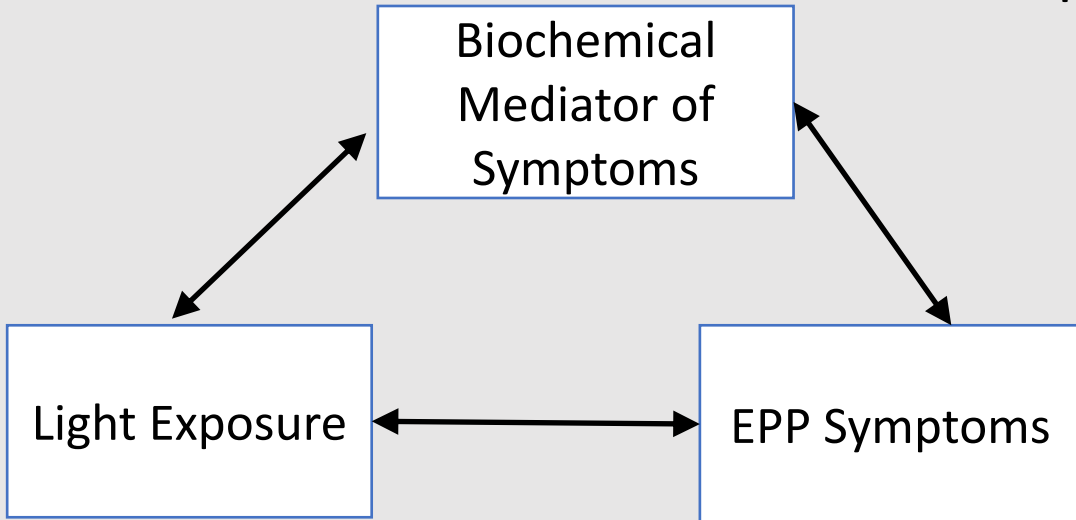
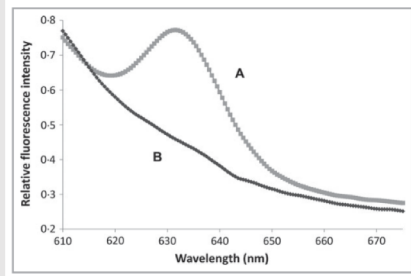
# The problem: severe painful photosensitivity

- Prolonged, untreatable pain
- Decreased quality of life
- Difficulty predicting and preventing symptoms
- No precise endpoints for clinical trials

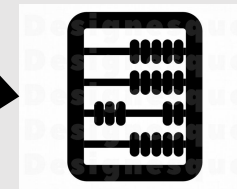
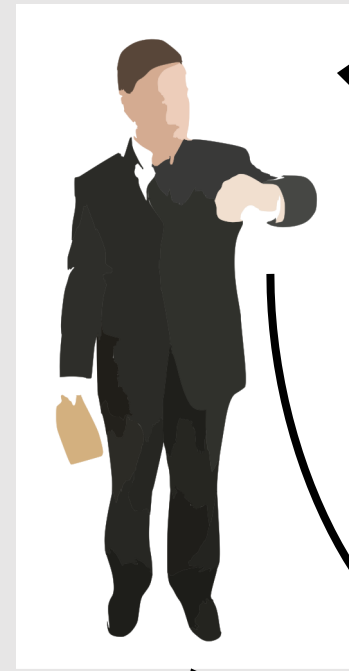
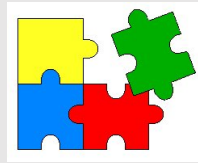


Erythropoietic protoporphyria (EPP)

# The solution: adapt and test technologies currently used for skin cancer



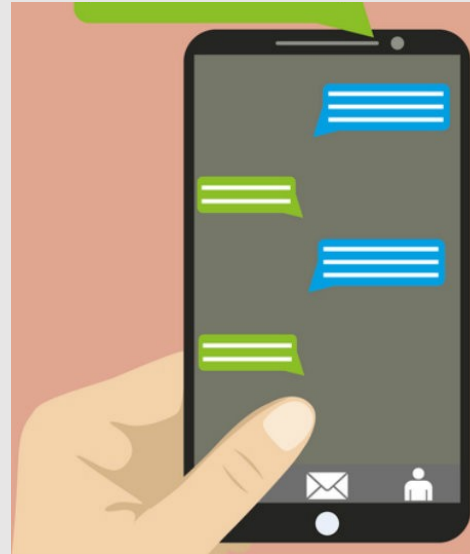
Integrate data



# The Products: Smartphone Based Digital Devices



SunSense Device:  
**Exposure**



Daily Text Surveys:  
**Symptoms**



Cutaneous Fluorescence  
Spectrometry:  
**Biochemical Response**



# The Technology: Adapting and Testing Devices

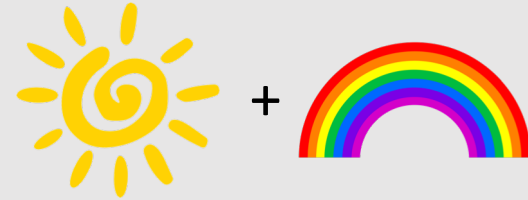
SunSense Pro

UV only

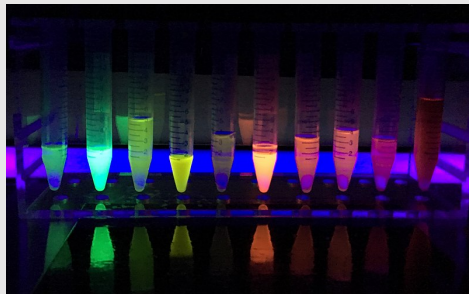


Sunsense Sensitive

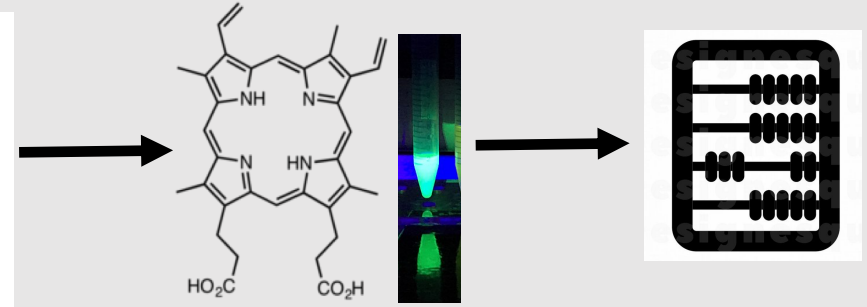
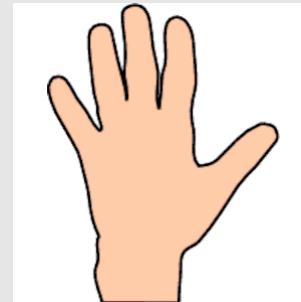
UV and visible light.



Fluorescence Spectrometry for  
Skin Screening



Cutaneous Fluorescence  
Spectrometry for EPP

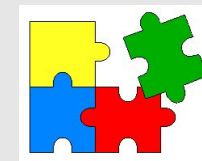


# Competing Approaches: Light Exposure Diary

Primary end point: cumulative number of hours in direct sunlight between 10am and 6pm without pain over a period of 6 months

1. EPP Monitoring										
1.1 Have you experienced any reactions to light today?    Yes <input type="checkbox"/> No <input type="checkbox"/>										
1.2 If 'yes', please indicate on the scale below how bad your pain was from this reaction:										
0	1	2	3	4	5	6	7	8	9	10
No Pain	Mild		Moderate			Severe			Worst Imaginable	
2. Time Spent Outdoors										
2.1 Did you spend any time outdoors today?    Yes <input type="checkbox"/> No <input type="checkbox"/>										
2.2 If 'yes', please enter the time period that you were in <u>direct sunlight</u> . (Each box represents 15 minutes)										
10:00	11:00	12:00	13:00	14:00	15:00	16:00	17:00	18:00		
█	█	█	█	█	█	█	█	█	█	█

Integrate  
data



# Traction

## Grants:

- Medicine Innovation Program Spark Grant
- Porphyrrias Consortium Funding

## Partnerships:

- SunSense
- Labby
- The Healthcare Transformation Lab at MGH
- The American Porphyria Foundation
- The Porphyrrias Consortium



HTL

Healthcare  
Transformation Lab





# My Team



## IDEA<sup>2</sup> Co-Mentors:

- Teresa Arroyo Gallego, PhD
- Judith Birkenfeld, PhD

## Mentors/supporting team:

- Irene Kochevar, PhD  
MGH, dermatology
- David Christiani, MD  
MGH, epidemiology and pulmonary
- Jared Conley, MD PhD  
Healthcare Transformation Lab
- Atle Brun, MD  
University of Bergen, EPP specialist
- Kristen Wheeden, MBA  
American Porphyria Foundation
- Candace Colbert, RN



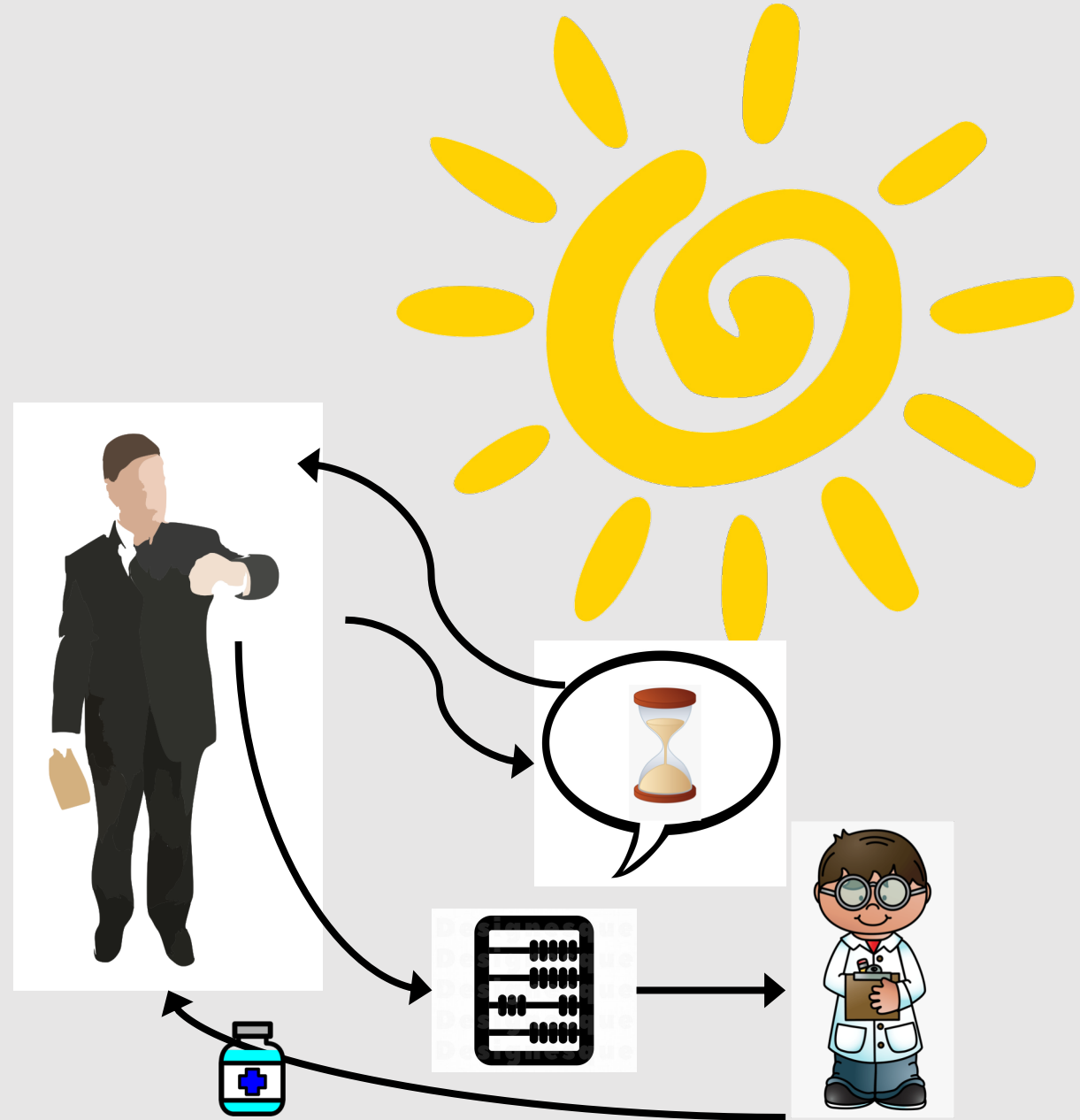
# Closing

## The goals:

- guide patient exposure
- quantitative endpoints for clinical trial data collection

## Ways this will help patients:

- improve quality of life
- facilitate the approval of new medications for EPP and other forms of photosensitivity



# Project update for IDEA<sup>2</sup>

1. **IRB:** Submitted IRB application, granted expedited review, resubmission. RISO review in process.
2. **Contracts:** Progress on contracts with SunSense and Labby
3. **Funding:** Funding for clinical coordinator through the Porphyrrias Consortium
4. **Logistics:** Worked out logistics regarding clinical coordinator, space to see patients, text/email RedCap surveys
5. **Team:** I have a team of appropriate mentors/advisors. Getting a biostats consultation. Working to identify a student or post-doc to help.

## Pivots:

1. SunSense Sensitive availability delayed to early March → initial troubleshooting with SunSense Pro & prototype testing with fluorescence spectrometry earlier
2. Afamelanotide approved by the FDA on Tuesday! → editing IRB to allow dosimeter before and after Afamelanotide use



Questions?