

# Stanford

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## Suzanne Pfeffer

Emma Pfeiffer Merner Professor of Medical Sciences  
Biochemistry

### Bio

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#### ACADEMIC APPOINTMENTS

- Professor, Biochemistry
- Member, Bio-X
- Faculty Fellow, Sarafan ChEM-H
- Member, Stanford Cancer Institute
- Member, Wu Tsai Neurosciences Institute

#### ADMINISTRATIVE APPOINTMENTS

- Assistant Professor, Stanford University School of Medicine-Biochemistry, (1986-1992)
- Associate Professor, Stanford University School of Medicine - Biochemistry, (1992-1998)
- Associate Chairman, Stanford University School of Medicine-Biochemistry, (1997-1998)
- Chairman, Stanford University School of Medicine - Biochemistry, (1998-2006)
- Professor, Stanford University School of Medicine-Biochemistry, (1998- present)
- Emma Pfeiffer Merner Professor of Medical Sciences, Stanford University School of Medicine, (2012- present)
- Chairman, Stanford University School of Medicine - Biochemistry, (2013-2019)

#### HONORS AND AWARDS

- Presidential Young Investigator Award, National Science Foundation (1988-1993)
- Fellow, American Association for the Advancement of Science (1992)
- Merit Award, National Institute of Diabetes and Digestive and Kidney Disorders (1999-2009)
- President, American Society for Cell Biology (2003)
- President, American Society for Biochemistry and Molecular Biology (2010-2012)
- Fellow, American Academy of Arts and Sciences (2013)
- Fellow, American Society for Cell Biology (2017)
- Senior Editor, eLife (2019-)

#### PROFESSIONAL EDUCATION

- A.B., U.C. Berkeley , Biochemistry (1978)
- Ph.D., U.C. San Francisco , Biochemistry (1983)
- Postdoctoral, U.C. San Francisco , Biochemistry (1984)

- Postdoctoral, Stanford University , Biochemistry (1985)

## LINKS

- Pfeffer Lab Site: <http://pfeffer.stanford.edu>

## Research & Scholarship

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### CURRENT RESEARCH AND SCHOLARLY INTERESTS

The long term goal of our research is to elucidate the molecular mechanisms by which proteins are targeted to specific and distinct compartments. We focus on the Rab GTPases that are master regulators of membrane trafficking. A molecular understanding of membrane traffic has broad implications for our understanding of growth control in cancer, receptor trafficking errors in heart disease, regulation of insulin secretion in diabetes and synaptic vesicle biogenesis and transport in neurological disorders.

LRRK2 that is hyperactive in some types of Parkinson's Disease specifically phosphorylates Rabs--we want to understand how this is linked to disease. We have found that phosphorylation of Rab10 blocks primary cilia formation in culture and in certain brain regions and we would like to understand how this leads to Parkinson's disease. We also study the NPC1 protein that is essential for cholesterol transport in humans and can lead to Niemann Pick C disease when mutated.

## Teaching

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### COURSES

#### 2022-23

- Advanced Cell Biology: BIO 214, BIOC 224, MCP 221 (Win)
- Biochemistry Mini-Course: BIOC 202 (Aut)

#### 2021-22

- Advanced Cell Biology: BIOC 224, MCP 221 (Win)
- Biochemistry Mini-Course: BIOC 202 (Aut)
- Frontiers in Biological Research: BIOC 215, DBIO 215, GENE 215 (Aut, Win, Spr)

#### 2020-21

- Advanced Cell Biology: BIO 214, BIOC 224, MCP 221 (Win)

#### 2019-20

- Advanced Cell Biology: BIO 214, BIOC 224, MCP 221 (Win)

### STANFORD ADVISEES

#### Doctoral Dissertation Reader (AC)

Martha Kahlson

#### Postdoctoral Faculty Sponsor

Ayan Adhikari, Ebsy Jaimon, Shahzad Khan, Yu En Lin, Sreeja Vijayan Nair

#### Doctoral Dissertation Advisor (AC)

Claire Chiang

### GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Biochemistry (Phd Program)

- Cancer Biology (Phd Program)
- Molecular and Genetic Medicine (Fellowship Program)
- Neurosciences (Phd Program)

## Publications

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### PUBLICATIONS

- **A feed-forward pathway drives LRRK2 kinase membrane recruitment and activation.** *eLife*  
Vides, E. G., Adhikari, A., Chiang, C. Y., Lis, P., Purlyte, E., Limouse, C., Shumate, J. L., Spinola-Lasso, E., Dhekne, H. S., Alessi, D. R., Pfeffer, S. R.  
2022; 11
- **LRRK2 phosphorylation of Rab GTPases in Parkinson's disease.** *FEBS letters*  
Pfeffer, S. R.  
2022
- **CRISPR screens for lipid regulators reveal a role for ER-bound SNX13 in lysosomal cholesterol export.** *The Journal of cell biology*  
Lu, A., Hsieh, F., Sharma, B. R., Vaughn, S. R., Enrich, C., Pfeffer, S. R.  
1800; 221 (2)
- **Pathogenic LRRK2 control of primary cilia and Hedgehog signaling in neurons and astrocytes of mouse brain** *ELIFE*  
Khan, S. S., Sobu, Y., Dhekne, H. S., Tonelli, F., Berndsen, K., Alessi, D. R., Pfeffer, S. R.  
2021; 10
- **Pathogenic LRRK2 regulates ciliation probability upstream of tau tubulin kinase 2 via Rab10 and RILPL1 proteins.** *Proceedings of the National Academy of Sciences of the United States of America*  
Sobu, Y., Wawro, P. S., Dhekne, H. S., Yeshaw, W. M., Pfeffer, S. R.  
2021; 118 (10)
- **LRRK2-phosphorylated Rab10 sequesters Myosin Va with RILPL2 during ciliogenesis blockade.** *Life science alliance*  
Dhekne, H. S., Yanatori, I., Vides, E. G., Sobu, Y., Diez, F., Tonelli, F., Pfeffer, S. R.  
2021; 4 (5)
- **Rab29 Fast Exchange Mutants: Characterization of a Challenging Rab GTPase.** *Methods in molecular biology (Clifton, N.J.)*  
Gomez, R. C., Vides, E. G., Pfeffer, S. R.  
2021; 2293: 19-25
- **Inter-domain dynamics drive cholesterol transport by NPC1 and NPC1L1 proteins.** *eLife*  
Saha, P. n., Shumate, J. L., Caldwell, J. G., Elghobashi-Meinhardt, N. n., Lu, A. n., Zhang, L. n., Olsson, N. E., Elias, J. E., Pfeffer, S. R.  
2020; 9
- **PPM1H phosphatase counteracts LRRK2 signaling by selectively dephosphorylating Rab proteins.** *eLife*  
Berndsen, K., Lis, P., Yeshaw, W. M., Wawro, P. S., Nirujogi, R. S., Wightman, M., Macartney, T., Dorward, M., Knebel, A., Tonelli, F., Pfeffer, S. R., Alessi, D. R.  
2019; 8
- **Membrane association but not identity is required for LRRK2 activation and phosphorylation of Rab GTPases.** *The Journal of cell biology*  
Gomez, R. C., Wawro, P., Lis, P., Alessi, D. R., Pfeffer, S. R.  
2019
- **NPC intracellular cholesterol transporter 1 (NPC1)-mediated cholesterol export from lysosomes.** *The Journal of biological chemistry*  
Pfeffer, S. R.  
2019; 294 (5): 1706-9
- **NPC intracellular cholesterol transporter 1 (NPC1)-mediated cholesterol export from lysosomes** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Pfeffer, S. R.  
2019; 294 (5): 1706-1709
- **Genome-wide interrogation of extracellular vesicle biology using barcoded miRNAs.** *eLife*

- Lu, A., Wawro, P., Morgens, D. W., Portela, F., Bassik, M. C., Pfeffer, S. R.  
2018; 7
- **Genome-wide interrogation of extracellular vesicle biology using barcoded miRNAs** *ELIFE*  
Lu, A., Wawro, P., Morgens, D. W., Portela, F., Bassik, M. C., Pfeffer, S. R.  
2018; 7
  - **LRRK2 and Rab GTPases** *BIOCHEMICAL SOCIETY TRANSACTIONS*  
Pfeffer, S. R.  
2018; 46: 1707–12
  - **LRRK2 and Rab GTPases.** *Biochemical Society transactions*  
Pfeffer, S. R.  
2018
  - **A pathway for Parkinson's Disease LRRK2 kinase to block primary cilia and Sonic hedgehog signaling in the brain.** *eLife*  
Dhekne, H. S., Yanatori, I., Gomez, R. C., Tonelli, F., Diez, F., Schule, B., Steger, M., Alessi, D. R., Pfeffer, S. R.  
2018; 7
  - **A pathway for Parkinson's Disease LRRK2 kinase to block primary cilia and Sonic hedgehog signaling in the brain** *ELIFE*  
Dhekne, H. S., Yanatori, I., Gomez, R. C., Tonelli, F., Diez, F., Schule, B., Steger, M., Alessi, D. R., Pfeffer, S. R.  
2018; 7
  - **Rab29 activation of the Parkinson's disease-associated LRRK2 kinase** *EMBO JOURNAL*  
Purlyte, E., Dhekne, H. S., Sarhan, A. R., Gomez, R., Lis, P., Wightman, M., Martinez, T. N., Tonelli, F., Pfeffer, S. R., Alessi, D. R.  
2018; 37 (1): 1–18
  - **Systematic proteomic analysis of LRRK2-mediated Rab GTPase phosphorylation establishes a connection to ciliogenesis** *ELIFE*  
Steger, M., Diez, F., Dhekne, H. S., Lis, P., Nirujogi, R. S., Karayel, O., Tonelli, F., Martinez, T. N., Lorentzen, E., Pfeffer, S. R., Alessi, D. R., Mann, M.  
2017; 6
  - **NPC1-mediated cholesterol export from lysosomes**  
Pfeffer, S. R.  
FEDERATION AMER SOC EXP BIOL.2017
  - **Rab GTPases: master regulators that establish the secretory and endocytic pathways** *MOLECULAR BIOLOGY OF THE CELL*  
Pfeffer, S. R.  
2017; 28 (6): 712-715
  - **Quantitative Measurement of Cholesterol in Cell Populations Using Flow Cytometry and Fluorescent Perfringolysin O.** *Methods in molecular biology (Clifton, N.J.)*  
Li, J., Lee, P. L., Pfeffer, S. R.  
2017; 1583: 85-95
  - **Lysosomal membrane glycoproteins bind cholesterol and contribute to lysosomal cholesterol export.** *eLife*  
Li, J., Pfeffer, S. R.  
2016; 5
  - **Clues to the mechanism of cholesterol transfer from the structure of NPC1 middle luminal domain bound to NPC2.** *Proceedings of the National Academy of Sciences of the United States of America*  
Li, X., Saha, P., Li, J., Blobel, G., Pfeffer, S. R.  
2016; 113 (36): 10079-10084
  - **Clues to NPC1-mediated cholesterol export from lysosomes.** *Proceedings of the National Academy of Sciences of the United States of America*  
Pfeffer, S. R.  
2016; 113 (29): 7941-7943
  - **Ezetimibe-sensitive cholesterol uptake by NPC1L1 protein does not require endocytosis** *MOLECULAR BIOLOGY OF THE CELL*  
Johnson, T. A., Pfeffer, S. R.  
2016; 27 (11): 1845-1852

- **Lipoprotein secretion: It takes two to TANGO.** *The Journal of cell biology*  
Pfeffer, S. R.  
2016
- **LAMP proteins bind cholesterol and contribute to NPC1-mediated cholesterol export from lysosomes.**  
Li, J., Pfeffer, S. R.  
AMER SOC CELL BIOLOGY.2016
- **Transport Vesicle Tethering at the Trans Golgi Network: Coiled Coil Proteins in Action.** *Frontiers in cell and developmental biology*  
Cheung, P. P., Pfeffer, S. R.  
2016; 4: 18-?
- **Glycosylation inhibition reduces cholesterol accumulation in NPC1 protein-deficient cells.** *Proceedings of the National Academy of Sciences of the United States of America*  
Li, J., Deffieu, M. S., Lee, P. L., Saha, P., Pfeffer, S. R.  
2015; 112 (48): 14876-14881
- **The Rab6-regulated KIF1C kinesin motor domain contributes to Golgi organization** *ELIFE*  
Lee, P. L., Ohlson, M. B., Pfeffer, S. R.  
2015; 4
- **Measuring Rab GTPase-Activating Protein (GAP) Activity in Live Cells and Extracts.** *Methods in molecular biology (Clifton, N.J.)*  
Nottingham, R. M., Pfeffer, S. R.  
2015; 1298: 61-71
- **Protein flexibility is required for vesicle tethering at the Golgi.** *eLife*  
Cheung, P. P., Limouse, C., Mabuchi, H., Pfeffer, S. R.  
2015; 4
- **Rab6 regulation of the kinesin family KIF1C motor domain contributes to Golgi tethering.** *eLife*  
Lee, P. L., Ohlson, M. B., Pfeffer, S. R.  
2015; 4
- **Molecular and Cellular Characterization of GCC185: A Tethering Protein of the Trans-Golgi Network.** *Methods in molecular biology (Clifton, N.J.)*  
Cheung, P. P., Pfeffer, S. R.  
2015; 1270: 179-190
- **Conformational flexibility of GCC185 is required for vesicle tethering at the trans Golgi.**  
Cheung, P., Limouse, C., Mabuchi, H., Pfeffer, S. R.  
AMER SOC CELL BIOLOGY.2014
- **A CULLINARY ride across the secretory pathway: more than just secretion** *TRENDS IN CELL BIOLOGY*  
Lu, A., Pfeffer, S. R.  
2014; 24 (7): 389-399
- **Mutant enzymes challenge all assumptions.** *eLife*  
Nottingham, R. M., Pfeffer, S. R.  
2014; 3
- **A Prize for Membrane Magic** *CELL*  
Pfeffer, S. R.  
2013; 155 (6): 1203-1206
- **Golgi-associated RhoBTB3 targets Cyclin E for ubiquitylation and promotes cell cycle progression** *JOURNAL OF CELL BIOLOGY*  
Lu, A., Pfeffer, S. R.  
2013; 203 (2): 233-250
- **Rab GTPase regulation of membrane identity** *CURRENT OPINION IN CELL BIOLOGY*  
Pfeffer, S. R.  
2013; 25 (4): 414-419

- **A nexus for receptor recycling.** *Nature cell biology*  
Pfeffer, S. R.  
2013; 15 (5): 446-448
- **Hopping rim to rim through the Golgi.** *eLife*  
Pfeffer, S. R.  
2013; 2
- **Ric1-Rgp1 Complex Is a Guanine Nucleotide Exchange Factor for the Late Golgi Rab6A GTPase and an Effector of the Medial Golgi Rab33B GTPase** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Pusapati, G. V., Luchetti, G., Pfeffer, S. R.  
2012; 287 (50): 42129-42137
- **Rab GTPase localization and Rab cascades in Golgi transport** *BIOCHEMICAL SOCIETY TRANSACTIONS*  
Pfeffer, S. R.  
2012; 40: 1373-1377
- **The 5-phosphatase OCRL mediates retrograde transport of the mannose 6-phosphate receptor by regulating a Rac1-cofilin signalling module** *HUMAN MOLECULAR GENETICS*  
van Rahden, V. A., Brand, K., Najm, J., Heeren, J., Pfeffer, S. R., Braulke, T., Kutsche, K.  
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- **Cargo carriers from the Golgi to the cell surface** *EMBO JOURNAL*  
Pfeffer, S. R.  
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- **TBC1D16 is a Rab4A GTPase activating protein that regulates receptor recycling and EGF receptor signaling** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*  
Goueli, B. S., Powell, M. B., Finger, E. C., Pfeffer, S. R.  
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- **RUTBC2 Protein, a Rab9A Effector and GTPase-activating Protein for Rab36** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Nottingham, R. M., Pusapati, G. V., Ganley, I. G., Barr, F. A., Lambright, D. G., Pfeffer, S. R.  
2012; 287 (27): 22740-22748
- **Ebola virus entry requires the host-programmed recognition of an intracellular receptor** *EMBO JOURNAL*  
Miller, E. H., Obernosterer, G., Raaben, M., Herbert, A. S., Deffieu, M. S., Krishnan, A., Ndungo, E., Sandesara, R. G., Carette, J. E., Kuehne, A. I., Ruthel, G., Pfeffer, S. R., Dye, et al  
2012; 31 (8): 1947-1960
- **Niemann-Pick type C 1 function requires luminal domain residues that mediate cholesterol-dependent NPC2 binding** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*  
Deffieu, M. S., Pfeffer, S. R.  
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- **RUTBC1 Protein, a Rab9A Effector That Activates GTP Hydrolysis by Rab32 and Rab33B Proteins** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Nottingham, R. M., Ganley, I. G., Barr, F. A., Lambright, D. G., Pfeffer, S. R.  
2011; 286 (38): 33213-33222
- **GCC185 plays independent roles in Golgi structure maintenance and AP-1-mediated vesicle tethering** *JOURNAL OF CELL BIOLOGY*  
Brown, F. C., Schindelheim, C. H., Pfeffer, S. R.  
2011; 194 (5): 779-787
- **Entry at the trans-Face of the Golgi** *COLD SPRING HARBOR PERSPECTIVES IN BIOLOGY*  
Pfeffer, S. R.  
2011; 3 (3)
- **How the Golgi works: A cisternal progenitor model** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*  
Pfeffer, S. R.

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Brown, F. C., Pfeffer, S. R.  
2010; 27 (8): 457-461
- **Membrane traffic Editorial overview** *CURRENT OPINION IN CELL BIOLOGY*  
Pfeffer, S. R., Novick, P. J.  
2010; 22 (4): 419-421
- **F1000 How Does Your Golgi Go?** *SCIENTIST*  
Pfeffer, S. R.  
2010; 24 (5): 65-66
- **Unconventional secretion by autophagosome exocytosis** *JOURNAL OF CELL BIOLOGY*  
Pfeffer, S. R.  
2010; 188 (4): 451-452
- **Recent advances in understanding Golgi biogenesis.** *F1000 biology reports*  
Pfeffer, S. R.  
2010; 2: 32-?
- **Two Rabs for exosome release** *NATURE CELL BIOLOGY*  
Pfeffer, S. R.  
2010; 12 (1): 3-4
- **Multiple routes of protein transport from endosomes to the trans Golgi network** *FEBS LETTERS*  
Pfeffer, S. R.  
2009; 583 (23): 3811-3816
- **Defining the boundaries: Rab GEFs and GAPs** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*  
Nottingham, R. M., Pfeffer, S. R.  
2009; 106 (34): 14185-14186
- **RhoBTB3: A Rho GTPase-Family ATPase Required for Endosome to Golgi Transport** *CELL*  
Espinosa, E. J., Calero, M., Sridevi, K., Pfeffer, S. R.  
2009; 137 (5): 938-948
- **Roles for Rab6, Arl1 and a novel Rho protein in GCC185-mediated vesicle tethering at the trans Golgi network**  
Pfeffer, S. R.  
FEDERATION AMER SOC EXP BIOL.2009
- **Rab9 regulation of the Rab GTPase activating protein, RUTBC1**  
Nottingham, R. M., Ganley, I. G., Barr, F. A., Lambright, D. G., Pfeffer, S. R.  
FEDERATION AMER SOC EXP BIOL.2009
- **Multiple Rab GTPase Binding Sites in GCC185 Suggest a Model for Vesicle Tethering at the Trans-Golgi** *MOLECULAR BIOLOGY OF THE CELL*  
Hayes, G. L., Brown, F. C., Haas, A. K., Nottingham, R. M., Barr, F. A., Pfeffer, S. R.  
2009; 20 (1): 209-217
- **WHAMMING into the Golgi** *DEVELOPMENTAL CELL*  
Hayes, G. L., Pfeffer, S. R.  
2008; 15 (2): 171-172
- **Team effort by TRAPP forces a nucleotide fumble** *CELL*  
Nottingham, R. M., Pfeffer, S. R.  
2008; 133 (7): 1141-1143
- **Rab and arl GTPase family members cooperate in the localization of the golgin GCC185** *CELL*  
Burguete, A. S., Fenn, T. D., Brunger, A. T., Pfeffer, S. R.

2008; 132 (2): 286-298

- **A syntaxin 10-SNARE complex distinguishes two distinct transport routes from endosomes to the trans-Golgi in human cells** *JOURNAL OF CELL BIOLOGY*  
Ganley, I. G., Espinosa, E., Pfeffer, S. R.  
2008; 180 (1): 159-172
- **A tribute to Arthur Kornberg 1918-2007** *NATURE STRUCTURAL & MOLECULAR BIOLOGY*  
Fuller, R. S., Bambara, R. A., Baker, T., Funnell, B., Wahle, E., O'Donnell, M., Kaiser, D., Skarstad, K., Konforti, B., Maki, S., Katayama, T., Sekimizu, K., Weiner, et al  
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Pfeffer, S. R.  
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Reddy, J. V., Ganley, I. G., Pfeffer, S. R.  
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- **A functional role for the GCC185 Golgin in mannose 6-phosphate receptor recycling** *MOLECULAR BIOLOGY OF THE CELL*  
Reddy, J. V., Burguete, A. S., Sridevi, K., Ganley, I. G., Nottingham, R. M., Pfeffer, S. R.  
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- **Lab management: insights for the new investigator** *NATURE IMMUNOLOGY*  
Haynes, L., Pfeffer, S., Boss, J. M., Kavathas, P., Kuchroo, V.  
2006; 7 (9): 895-897
- **Cholesterol accumulation sequesters Rab9 and disrupts late endosome function in NPC1-deficient cells** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Ganley, I. G., Pfeffer, S. R.  
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- **TIP47 is a key effector for Rab9 localization** *JOURNAL OF CELL BIOLOGY*  
Aivazian, D., Serrano, R. L., Pfeffer, S.  
2006; 173 (6): 917-926
- **Misincorporation proton-alkyl exchange (MPAX): engineering cysteine probes into proteins.** *Current protocols in protein science / editorial board, John E. Coligan ... [et al.]*  
Burguete, A. S., Harbury, P. B., Pfeffer, S. R.  
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- **Filling the Rab GAP** *NATURE CELL BIOLOGY*  
Pfeffer, S.  
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- **A model for rab GTPase localization** *Conference on Localization and Activation of Ras-Like GTPases*  
Pfeffer, S.  
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- **Structural clues to Rab GTPase functional diversity** *JOURNAL OF BIOLOGICAL CHEMISTRY*  
Pfeffer, S. R.  
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- **Purification and properties of Yip3/PRA1 as a Rab GDI displacement factor** *GTPASES REGULATING MEMBRANE TARGETING AND FUSION*  
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- **Purification and analysis of TIP47 function in Rab9-dependent mannose 6-phosphate receptor trafficking** *GTPASES REGULATING MEMBRANE TARGETING AND FUSION*  
Burguete, A. S., Sivars, U., Pfeffer, S.  
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Ganley, I. G., CARROLL, K., Bittova, L., Pfeffer, S.  
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Pfeffer, S. R.  
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Pfeffer, S. R.  
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